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As filed with the Securities and Exchange Commission on , 2013

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM S-1

REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

PTC THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**2834**	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

100 Corporate Court
South Plainfield, New Jersey 07080
(908) 222-7000
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Stuart W. Peltz, Ph.D.
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100 Corporate Court
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(908) 222-7000
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Approximate date of commencement of proposed sale to the public:
As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. o

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o _____

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o _____

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o _____

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer o	Accelerated filer o	Non-accelerated filer ☒ (Do not check if a smaller reporting company)	Smaller reporting company o

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities To Be Registered	Proposed Maximum Aggregate Offering Price(1)	Amount of Registration Fee(2)
Common Stock, $0.001 par value per share	$85,000,000(1)	$11,594

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.

(2) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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Subject to completion, dated , 2013

The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Prospectus

shares

Common stock

This is an initial public offering of common stock by PTC Therapeutics, Inc. We are selling shares of our common stock. The estimated initial public offering price is between $ and $ per share.

Prior to this offering, there has been no public market for our common stock. We have applied for listing of our common stock on the Nasdaq Global Market under the symbol "PTCT".

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012, and as such, have elected to comply with certain reduced public company reporting requirements.

Investing in our common stock involves a high degree of risk. See "Risk factors" beginning on page 11.

	Per share	Total
Initial public offering price	$	$
Underwriting discounts and commissions	$	$
Proceeds to PTC Therapeutics, Inc., before expenses	$	$

We have granted the underwriters an option for a period of 30 days to purchase up to additional shares of common stock to cover any over-allotments. The underwriters can exercise this right at any time within 30 days after the date of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to investors on or about , 2013.

J.P. Morgan **Credit Suisse**

Cowen and Company

Wedbush PacGrow Life Sciences

The date of this prospectus is , 2013

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We have not authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock. Our business, financial conditions, results of operations and prospects may have changed since that date.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

Prospectus summary

This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the "Risk factors" section and our financial statements and the related notes appearing at the end of this prospectus, before making an investment decision.

Our company overview

We are a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes. While our discovery programs are directed at targets in multiple therapeutic areas, we are focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. Our lead product candidate is ataluren for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. We have retained worldwide commercialization rights to ataluren for all indications in all territories. Ataluren is in late stage clinical development for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, and cystic fibrosis caused by nonsense mutations, or nmCF. There are currently no marketed therapies approved to treat the underlying cause of nmDMD or nmCF. The European Medicines Agency, or EMA, has designated ataluren as an orphan medicinal product and the U.S. Food and Drug Administration, or FDA, has granted orphan drug designation to ataluren for the treatment of both nmDMD and nmCF.

We are initiating a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD. We expect to dose the first patient in this trial in the first half of 2013. In October 2012, we submitted a marketing authorization application, or MAA, to the EMA for conditional approval of ataluren for the treatment of nmDMD. We are also planning a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. We plan to initiate this trial in the second half of 2013, subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design. We have completed a Phase 2b clinical trial of ataluren for the treatment of nmDMD and a Phase 3 clinical trial of ataluren for the treatment of nmCF. We did not achieve the primary efficacy endpoint in either trial with the pre-specified level of statistical significance. However, we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that ataluren was active and showed clinically meaningful improvements over placebo in these trials. In addition, we believe that our experience in these completed clinical trials has allowed us to enhance the designs of our confirmatory Phase 3 clinical trials and improve our likelihood of success in these trials. Ataluren has been generally well tolerated in all of our clinical trials to date.

The letters "PTC" in our corporate name are an acronym for post-transcriptional control processes, which are the regulatory events that occur in cells after a messenger RNA, or mRNA, molecule is copied, or transcribed, from DNA. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. Nonsense mutations create a premature stop signal in the translation of the genetic code contained in mRNA and prevent the production of full-length, functional proteins. The absence or overproduction of specific proteins can cause disease. We apply proprietary technologies and our extensive knowledge of post-transcriptional control processes in our drug discovery and development activities. We discovered ataluren by applying our technologies to identify molecules that promote or enhance the suppression of nonsense mutations.

In addition to ataluren, we have a pipeline of product candidates that are in preclinical development. Our preclinical and discovery programs are focused on the development of new treatments for multiple

therapeutic areas, including neuromuscular disease, oncology and infectious disease. We have discovered all of our compounds currently under development using our proprietary technologies. We plan to develop these compounds both on our own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For example, we currently are collaborating with F. Hoffman-La Roche Ltd and Hoffman-La Roche, Inc. and the Spinal Muscular Atrophy Foundation for the development and commercialization of compounds for the treatment of spinal muscular atrophy.

Ataluren

Ataluren is a novel, orally administered small-molecule compound that targets nonsense mutations. We are developing ataluren for the treatment of genetic disorders in which a nonsense mutation is the cause of the disease. Genetic tests are available for many genetic disorders, including Duchenn muscular dystrophy and cystic fibrosis, to determine if the underlying cause is a nonsense mutation.

We believe that ataluren interacts with the ribosome, which is the component of the cell that decodes the mRNA molecule and manufactures proteins, to enable the ribosome to read through premature nonsense stop signals on mRNA and allow the cell to produce a full-length, functional protein. We believe that a drug with a mechanism of action that allows the ribosome to read through premature stop signals without affecting the normal terminatio of protein synthesis may be able to overcome the effects of nonsense mutations.

Ataluren is administered orally as granules mixed with permitted liquids or semi-solid foods, such as milk, water, applesauce or yogurt. We designed this formulation because children comprise a significant portion of the patient population for ataluren and often have difficulty swallowing pills or capsules. Ataluren is manufactured in reliable and reproducible synthetic processes from readily available starting materials.

Ataluren for nmDMD

Muscular dystrophies are genetic disorders involving progressive muscle wasting and weakness. Duchenne muscular dystrophy is the most common and one of the most severe types of muscular dystrophy. Duchenne muscular dystrophy occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Based on information from the American Journal of Medical Genetics, we estimate that a nonsense mutation is the cause of Duchenne muscular dystrophy in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union. There is currently no marketed therapy approved for the treatment of the underlying cause of Duchenne muscular dystrophy. Currently available treatments for Duchenne muscular dystrophy are only palliative.

We are currently enrolling trial sites for a multicenter, randomized, double-blind, placebo controlled Phase 3 clinical trial to evaluate the efficacy and safety of ataluren in patients with nmDMD. We plan to conduct this trial in approximately 220 patients at investigational sites worldwide. The primary objective of this trial will be to evaluate the effect of ataluren on ambulation as measured by mean change in distance walked during a 6-minute walk test, which we refer to as 6-minute walk distance. Based on our plan to dose the first patients in this trial in the first half of 2013 and our estimates regarding patient enrollment, we expect to complete this trial and have initial, top-line data available in mid-2015.

The trial protocol specifies the following key inclusion criteria for patients enrolling in this trial:

- the patient must be seven through 16 years of age;

- at baseline, the patient must walk no more than 80% of predicted 6-minute walk distance compared to healthy boys matched for age and height but have the ability to walk at least 150 meters during the 6-minute walk test; and

- the patient must have used systemic corticosteroids for a minimum of six months prior to start of treatment.

The study population and outcome measures that we are using in our confirmatory Phase 3 clinical trial are based on, and reflect our analysis of the results of, our completed Phase 2b clinical trial for the treatment of nmDMD, including data regarding disease progression, referred to as natural histor data, and a post-hoc, retrospective subgroup analysis of patients who would meet the enrollment criteria for our confirmatory Phase 3 clinical trial. This retrospective subgroup analysis showed a much larger treatment effect in mean change in 6-minute walk distance between ataluren and placebo in thi subgroup than in the overall population included in the Phase 2b clinical trial. In light of this natural history data and retrospective subgroup analysis, our confirmatory Phase 3 clinical trial will focus on patients in the decline phase of the disease based on age and baseline 6-minute walk distance. The intent of focusing on patients in the decline phase of the disease is to enhance the demonstration of ataluren's effect to slow decline in walking ability. I addition, we believe that by only enrolling patients who are treated with systemic corticosteroids, the variability of 6-minute walk distance results will be reduced.

In October 2012, we submitted an MAA to the EMA for conditional approval of ataluren for the treatment of nmDMD. Although there is substantial risk that the EMA will not grant us this conditional approval, such approval would permit us to market ataluren in the European Union for treatment of nmDMD prior to completion of our confirmatory Phase 3 clinical trial for this indication. We plan to complete our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD before applying for marketing approval from the FDA. In designing our confirmatory Phase 3 clinical trial for the treatment of nmDMD, we have sought to reflect the views expressed by both the FDA and the EMA in our discussions with these regulatory authorities We expect that these trial results, if favorable, could serve as the basis for full approval by the FDA and EMA of ataluren for the treatment of nmDMD.

Ataluren for nmCF

Cystic fibrosis is among the most common life-threatening genetic disorders worldwide. Cystic fibrosis is caused by defects in a single gene known as the cystic fibrosis transmembrane conductance regulator, or CFTR. Based on information from the Cystic Fibrosis Foundation, we estimate that nonsense mutations are the cause of cystic fibrosis in approximately 10% of patients, or approximately 3,000 patients in the United States and approximately 3,700 to 4,200 patients in the European Union. There is currently no marketed therapy approved to correct defective CFTR production and function in patients with nmCF. For nmCF patients, available treatments do not address the underlying cause of the disease and are designed only to alleviate the symptoms of the disease.

We are planning a multicenter, randomized, double-blind, placebo controlled Phase 3 clinical trial to evaluate the efficacy and safety of ataluren in approximately 210 patients with cystic fibrosis caused by a nonsense mutation as confirmed by gene sequencing. We expect that the primary objective of this trial will be to evaluate the effect of ataluren on pulmonary function as measured by relative change in percent of predicted forced expiratory volume in one second, or FEV_1. FEV_1 is a measure of the volume of air that has been exhaled at the end of the first second of forced expiration. Percent of predicted FEV_1, or %-predicted FEV_1, is based on a comparison to healthy individuals matched for age, height and gender.

We expect to require that patients in this trial be at least six years of age and have %-predicted FEV_1 within a specified range, sweat chloride in excess of a specified level as evidence of the severity of the disease and documentation of a nonsense mutation in at least one copy of the CFTR gene. We expect to exclude patients from the trial if, among other reasons, they are receiving chronic inhaled aminoglycoside antibiotics.

We selected the enrollment criteria for our confirmatory Phase 3 clinical trial in part based on a subgroup analysis of patients not receiving inhaled aminoglycoside antibiotics in our completed Phase 3 clinical trial for the treatment of nmCF. We believe that the inhaled antibiotic tobramycin interfered with ataluren's mechanism of action. For the subgroup of patients not receiving chronic inhaled aminoglycoside antibiotics, there was a substantial difference in mean relative changes from baseline in %-predicted FEV_1 at the end of the trial favoring ataluren in comparison with placebo. In contrast, patients that received chronic inhaled aminoglycoside antibiotics and ataluren did not exhibit a difference compared to patients that received chronic inhaled aminoglycoside antibiotics and placebo.

We have received scientific advice from the EMA regarding the possibility of submitting an MAA for conditional approval of ataluren for the treatment of nmCF and the protocol design of a post-approval confirmatory trial. There also is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of nmCF. In addition, we have begun discussions with the FDA regarding the clinical development design options which would have the potential to support the submission of a new drug application with the FDA. Our goal in these continuing discussions is to achieve consensus between the EMA and the FDA that a single placebo controlled Phase 3 trial can serve as the basis for full approval of ataluren to treat nmCF in both the European Union and the United States.

Our strategy

Our goal is to become a leading biopharmaceutical company focused on discovering, developing and commercializing small-molecule therapeutics that target post-transcriptional control processes and address disorders, particularly in the orphan and ultra-orphan areas, with high unmet medical needs. To achieve our goal, we are pursuing the following strategies:

- Complete clinical development and seek marketing approvals for ataluren for the treatment of nmDMD and nmCF.
- Commercialize ataluren through our own focused, specialized sales force initially in the European Union and the United States and, eventually, in other key territories.
- Explore additional, strategically attractive indications for ataluren based on the large number of genetic disorders caused by nonsense mutations.
- Advance the development of our preclinical product candidates and discover and develop additional small molecules that alter post-transcriptional control processes in a broad range of indications.
- Seek third party grants and support and selectively establish strategic alliances.

Risks associated with our business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- We currently depend heavily on the success of ataluren. Our ability to generate product revenues, which may not occur for several years, if eve will depend heavily on the successful development and commercialization of ataluren for either or both of nmDMD and nmCF. There is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of either nmDMD or nmCF.

- Clinical trials of ataluren or any of our other product candidates may not be successful. If we are unable to obtain required marketing approvals for, commercialize, obtain and maintain patent protection for or gain market acceptance by physicians, patients and third-party payors of atalure or any of our other product candidates, or experience significant delays in doing so, our business will be materially harmed and our ability to generate revenue will be materially impaired.

- Our scientific approach focusing on the discovery and development of product candidates that target post-transcriptional control processes is unproven and may not result in the development of commercially viable drugs that safely and effectively treat genetic disorders or other diseases.

- Our current and any future collaborations with third parties for the development and commercialization of our product candidates may not be successful.

- We have a limited operating history. We currently have no commercial products and we have not received marketing approval for any product candidate.

- We have incurred significant operating losses since inception and may need substantial additional funding. We expect to incur significant expenses and increasing operating losses for at least the next several years. As of December 31, 2012, we had an accumulated deficit of $277.2 million.

Our corporate information

Our executive offices are located at 100 Corporate Court, South Plainfield, New Jersey 07080, and our telephone number is (908) 222-7000. Our website address is *www.ptcbio.com*. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

In this prospectus, unless otherwise stated or the context otherwise requires, references to "PTC," "PTC Therapeutics," "we," "us," "our" and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its consolidated subsidiaries. The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third party research, surveys and studies are reliable, we have not independently verified such data. This prospectus also includes data based on our own internal estimates and research. While we believe that our internal company research is reliable and that our internal estimates are reasonable, no independent source has verified such research or estimates.

Implications of being an emerging growth company

As a company with less than $1 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

The offering

Common stock offered by us	shares
Common stock to be outstanding after this offering	shares
Over-allotment option	The underwriters have an option for a period of 30 days to purchase up to additional shares of our common stock t cover over-allotments.
Use of proceeds	We intend to use the net proceeds from this offering to fund the clinical development of ataluren for the treatment of nmDMD and nmCF, to seek marketing approval in the European Union and the United States for ataluren for these indications, to pursue the development of ataluren for additional indications, to fund our other research and development programs and for working capital and other general corporate purposes. See "Use of proceeds" for more information.
Risk factors	You should read the "Risk factors" section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Proposed NASDAQ Global Market symbol	"PTCT"

The number of shares of our common stock to be outstanding after this offering is based on 739,850 shares of our common stock outstanding as of March 15, 2013 and 13,795,956 additional shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering.

The number of shares of our common stock to be outstanding after this offering excludes:

- 46,638 shares of our common stock issuable upon the exercise of stock options outstanding as of March 15, 2013, at a weighted-average exercise price of $ per share;
- 20,332 additional shares of our common stock available for future issuance as of March 15, 2013 under our 2009 equity and long term incentive plan;
- additional shares of our common stock that will be available for future issuance, as of the closing of this offering, under our 2013 public company stock incentive plan; and
- 17,012 shares of our common stock issuable upon the exercise of warrants outstanding as of March 15, 2013, at a weighted-average exercise price of $137.92 per share.

Unless otherwise indicated, all information in this prospectus assumes:

- no exercise of the outstanding options or warrants described above;

- no exercise by the underwriters of their option to purchase up to additional shares of our common stock to cover over-allotments;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 13,795,956 shares of our common stock upon the closing of this offering;
- the warrants outstanding as of March 15, 2013 to purchase an aggregate of 16,368 shares of our series five junior preferred stock, at an exercise price of $128 per share, automatically become warrants to purchase 16,368 shares of our common stock at an exercise price of $128 per share upon the closing of this offering; and
- the restatement of our certificate of incorporation and the amendment and restatement of our bylaws upon the closing of this offering.

In addition, unless otherwise indicated, all information in this prospectus gives effect to a one-for-120 reverse stock split of our common stock that was effected on March 7, 2013.

Summary financial data

You should read the following summary financial data together with our financial statements and the related notes appearing at the end of this prospectus and the "Selected financial data" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus. We have derived the statements of operations data for the years ended December 31, 2011 and 2012 from our audited financial statements included in this prospectus. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

	Year ended December 31,	
	2011	**2012**
(in thousands, except share and per share data)		
Statement of operations data		
Revenues:		
Collaboration revenue	$ 98,961	$ 28,779
Grant revenue	6,451	5,167
Total revenues	105,412	33,946
Operating expenses:		
Research and development	58,677	46,139
General and administrative	16,153	14,615
Total operating expenses	74,830	60,754
Income (loss) from operations	30,582	(26,808)
Interest expense, net	(2,444)	(1,210)
Other income, net	461	1,783
Income (loss) before tax benefit	28,599	(26,235)
Income tax benefit	2,306	—
Net income (loss)	30,905	(26,235)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	159,954
Less beneficial conversion charge	—	(378)
Net income attributable to common stockholders	$ 30,905	$ 133,341
Net income per share(1)		
Basic	$ 23.95	$ 219.76
Diluted	$ 4.55	$ 42.50
Weighted-average shares outstanding:		
Basic	1,089	3,328
Diluted	5,729	17,205

(1) See Note 8 to our financial statements appearing at the end of this prospectus regarding the calculation of net income per share.

As of December 31, 2012	Actual	Pro forma(1)	Pro forma as adjusted(2)
		(in thousands)	
Balance sheet data			
Cash, cash equivalents and short-term investments	$ 2,726	$	$
Working capital (deficit)	(23,564)		
Total assets	13,072		
Long-term debt, including current portion	4,883		
Convertible preferred stock	80,824		
Accumulated deficit	(277,225)		
Total stockholders' deficit	(99,641)		

(1) The pro forma balance sheet data give effect to the automatic conversion of all outstanding shares of our preferred stock, including shares of our series four senior preferred stock that we issued on March 7, 2013, into an aggregate of 13,795,956 shares of our common stock upon the closing of this offering.

(2) The pro forma as adjusted balance sheet data give further effect to our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents and short-term investments, working capital, total assets and total stockholders' equity by approximately $, assuming that the number of shares offered by us as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Risk factors

Investing in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks related to our financial position and need for additional capital

We have incurred significant losses since our inception. We expect to incur losses for at least the next several years and may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. As of December 31, 2012, we had an accumulated deficit of $277.2 million. To date, we have financed our operations primarily through private placements of our preferred stock, collaborations, bank debt and convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We have devoted substantially all of our efforts to research and development, including clinical trials. We have not completed development of any drugs. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. The net losses we incur may fluctuate significantly from quarter to quarter.

We anticipate that our expenses will increase substantially in connection with initiating and completing confirmatory Phase 3 clinical trials for our lead product candidate, ataluren, for the treatment of patients with Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, and patients with cystic fibrosis caused by nonsense mutations, or nmCF, and seeking marketing approval for ataluren for these indications in the European Union and the United States. We have submitted a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, for conditional approval of ataluren for the treatment of nmDMD. Subject to the conclusion of our ongoing discussions with regulatory authorities regarding the proposed design of our planned Phase 3 clinical trial, we also plan to pursue conditional approval of ataluren for the treatment of nmCF in the European Union. EMA conditional approval would permit us to market ataluren in the European Union for treatment of the applicable indication prior to completion of the confirmatory Phase 3 clinical trial for that indication. If we obtain marketing approval of ataluren for either nmDMD or nmCF, we also expect to incur significant sales, marketing, distribution and manufacturing expenses. The timing of commercialization expenses for ataluren depends in part on whether we receive conditional approval for ataluren for either or both of nmDMD and nmCF.

In addition, our expenses will increase if and as we:

- initiate or continue the research and development of ataluren for additional indications and of our other product candidates;
- seek to discover and develop additional product candidates;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Our ability to generate profits from operations and remain profitable depends on our ability to successfully develop and commercialize drugs that generate significant revenue. Based on our current plans, we do not expect to generate significant revenue unless and until we obtain marketing approval for, and

commercialize, ataluren for the treatment of nmDMD or nmCF. This will require us to be successful in a range of challenging activities, including:

- obtaining approval to market ataluren for the treatment of either or both of nmDMD and nmCF;
- successfully initiating and completing confirmatory Phase 3 clinical trials of ataluren for the treatment of either or both of nmDMD and nmCF;
- protecting our rights to our intellectual property portfolio related to ataluren;
- contracting for the manufacture of commercial quantities of ataluren;
- negotiating and securing adequate reimbursement from third-party payors for ataluren; and
- establishing sales, marketing and distribution capabilities to effectively market and sell ataluren in the European Union and the United States.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to generate profits from operations. Even if we do generate profits from operations, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to generate profits from operations and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we initiate and continue confirmatory Phase 3 clinical trials of ataluren for the treatment of nmDMD and nmCF, continue our research activities in our preclinical programs and initiate clinical development of other product candidates. In addition, if we obtain regulatory approval for ataluren or any of our other product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, short-term investments and research funding that we expect to receive under our collaborations, will be sufficient to enable us to fund our operating expenses, debt service obligations and capital expenditure requirements until . We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. This estimate assumes, among other things, that we do not receive conditional approval to market ataluren for nmDMD or nmCF in the European Union prior to completing a confirmatory Phase 3 trial for the applicable indication and, as a result, that we do not incur significant related commercialization expenses prior to such time. Our future capital requirements will depend on many factors, including:

- the progress and results of confirmatory Phase 3 clinical trials of ataluren for nmDMD and nmCF;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for ataluren for additional indications and for our other product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs, timing and outcome of regulatory review of ataluren and our other product candidates;

- the costs and timing of commercialization activities, including product sales, marketing, distribution and manufacturing, for any of our product candidates that receive marketing approval;
- subject to receipt of marketing approval, revenue received from commercial sales of ataluren or any of our other product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products and technologies; and
- our ability to establish and maintain collaborations.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we are not planning to have commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our stockholders, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates and marketing, distribution or licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to organizing and staffing our company, developing and securing our technology, raising capital and undertaking preclinical studies and clinical trials of our product candidates. We have not yet demonstrated our ability to successfully complete development of any product candidates, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Risks related to the development and commercialization of our product candidates

We depend heavily on the success of our lead product candidate, ataluren, which we are developing for nmDMD and nmCF. All of our other product candidates are still in preclinical development. If we are unable to commercialize ataluren, or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the development of ataluren for nmDMD and nmCF. Our ability to generate product revenues, which may not occur for several years, if ever, will depend heavily on the successful development and commercialization of ataluren. The success of ataluren will depend on a number of factors, including the following:

- successful completion of confirmatory Phase 3 clinical trials of ataluren;
- receipt of marketing approvals for ataluren in the European Union and the United States, including possible receipt of conditional approval to market ataluren in the European Union prior to completion of confirmatory Phase 3 clinical trials;
- establishing commercial manufacturing arrangements with third-party manufacturers;
- launching commercial sales of ataluren, if and when approved, whether alone or in collaboration with others;
- acceptance of ataluren, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- a continued acceptable safety profile of ataluren following approval;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and
- protecting our rights in our intellectual property portfolio.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize ataluren, which would materially harm our business.

If clinical trials of our product candidates, such as our confirmatory Phase 3 clinical trials of ataluren, fail to demonstrate safety and efficacy to the satisfaction of the EMA or the U.S. Food and Drug Administration, or FDA, or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of ataluren or any other product candidate.

In connection with obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product

candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

For example, we did not achieve the primary efficacy endpoint with the pre-specified level of statistical significance in a Phase 2b clinical trial of ataluren for the treatment of nmDMD that we completed in 2009 or in a Phase 3 clinical trial of ataluren for the treatment of nmCF that we completed in 2011. Although we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that ataluren was active and showed clinically meaningful improvements over placebo in these trials, we may similarly fail to achieve the primary efficacy endpoint in confirmatory Phase 3 clinical trials of ataluren for these indications. Some of our favorable statistical data also are based on nominal p-values that reflect only one particular comparison when more than one comparison is possible, such as when two active treatments are compared to placebo or when two or more subgroups are analyzed. Regulatory authorities typically give greatest weight to results from pre-specified analyses and adjusted p-values and less weight to results from post-hoc, retrospective analyses and nominal p-values. If the results of our confirmatory Phase 3 clinical trials are not favorable, we may need to conduct additional clinical trials at significant cost or altogether abandon development of ataluren for either or both of nmDMD and nmCF. We also did not achieve the primary objective in one of four prior Phase 2 clinical trials that we conducted for ataluren for the treatment of nmCF in which we measured change in chloride conductance in nasal cells over the course of treatment.

In addition, in connection with our preparation to apply for conditional approval of ataluren for the treatment of nmDMD, the EMA informed us in scientific advice that it provided in May 2012 that although ataluren falls within the scope of the regulation for conditional approval for this indication, it appeared unlikely that a positive risk-benefit ratio for ataluren can be concluded primarily based on the results of our completed Phase 2b clinical trial. In addition, the EMA questioned whether it is practical to believe that a confirmatory Phase 3 clinical trial for this indication could be continued if the EMA grants conditional approval, which would reduce the likelihood that we could provide comprehensive data following conditional approval. As a result, there is substantial risk that the EMA will not grant us the conditional approval for which we have applied and will not consider approval of ataluren for the treatment of nmDMD until we have completed a confirmatory Phase 3 clinical trial for this indication, which would delay the potential commercialization of this product candidate and our receipt of related revenues. We expect to face similar risks if we apply for conditional approval of ataluren for the treatment of nmCF prior to completing a confirmatory Phase 3 clinical trial for this indication. In particular, conditional approval of ataluren for the treatment of nmCF will depend on the EMA's assessment of the relative risks and benefits of conditional approval and our ability to provide comprehensive clinical data from a post-approval confirmatory trial.

Further, although we have had discussions with the FDA regarding our proposed confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF, the FDA may not consider our proposed trial design acceptable. This could potentially cause us to conduct more than one confirmatory clinical trial or could delay or prevent our ability to receive marketing approval for this indication.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;

- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;

- we may be unable to enroll a sufficient number of patients in our trials to ensure adequate statistical power to detect any statistically significant treatment effects;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of ataluren or any other product candidate that we develop beyond those that we contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

In particular, it is possible that the EMA or the FDA may not consider the results of our confirmatory Phase 3 clinical trial of ataluren for nmDMD or nmCF, once completed and even if successful, to be sufficient for approval of ataluren for such indication. The FDA typically requires two adequate and well-controlled pivotal clinical trials to support marketing approval of a product candidate for a particular indication. The EMA or the FDA could determine that the results of our trials are not sufficiently robust, are subject to confounding factors or are not adequately supported by other trial endpoints.

Our product development costs will increase if we experience delays in testing or marketing approvals. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is increased risk that the outcome of our clinical trials will not be favorable.

There are no marketed therapies approved to treat the underlying cause of nmDMD or nmCF. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat either of these diseases. As a result, the design and conduct of clinical trials for these diseases, particularly for drugs to address the underlying nonsense mutations causing these diseases in some subsets of patients, is subject to increased risk.

Prior to our conducting the Phase 2b clinical trial of ataluren for nmDMD, there was no established precedent for an appropriate trial design to evaluate the efficacy of ataluren for nmDMD and little clinical experience in the methodologies used to analyze the resulting data. Although we believe that we now understand the issues with the pre-specified statistical analyses of our Phase 2b trial results and that we have designed our confirmatory Phase 3 clinical trial of ataluren for nmDMD in an appropriate fashion, we may nonetheless experience similar or other unknown complications with our confirmatory Phase 3 clinical trial because of the limited clinical experience in this indication. As a result, we may not achieve the pre-specified endpoint with statistical significance in our confirmatory Phase 3 clinical trial, which would make approval of ataluren for this indication unlikely. Among other endpoints in our confirmatory Phase 3 clinical trial of ataluren for nmDMD, the trial protocol includes two secondary endpoints that have not been used previously as outcome measures in published therapeutic clinical trials. These endpoints, in particular, may produce results that are unpredictable or inconsistent with other trial results.

With regard to nmCF, we believe that we now understand subgroup effects that we observed in our completed Phase 3 clinical trial and that we have designed our confirmatory Phase 3 clinical trial of ataluren for nmCF to take these effects into account. However, we may nonetheless experience unknown complications with our confirmatory Phase 3 clinical trial. As a result, we may not achieve the pre-specified endpoint with statistical significance in our confirmatory Phase 3 clinical trial, which would make approval of ataluren for this indication unlikely.

If we experience delays or difficulties in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates, including our confirmatory Phase 3 clinical trials of ataluren, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. For example, both nmDMD and nmCF are characterized by relatively small patient populations, which could result in slow enrollment of clinical trial participants. In addition, our competitors have ongoing clinical trials for product candidates that could be competitive with our product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is affected by other factors including:

- severity of the disease under investigation;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of the company to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients in our confirmatory Phase 3 clinical trials of ataluren or any of our other clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

If serious adverse or inappropriate side effects are identified during the development of ataluren or any other product candidate, we may need to abandon or limit our development of that product candidate.

All of our product candidates are in clinical or preclinical development and their risk of failure is high. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval. If our product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound.

For example, although we did not observe a pattern of liver enzyme elevations in our Phase 2 or Phase 3 clinical trials of ataluren, we did observe modest elevations of liver enzymes in some subjects in one of our Phase 1 clinical trials. These elevated enzyme levels did not require cessation of ataluren administration, and enzyme levels typically normalized after completion of the treatment phase. We did not observe any increases in bilirubin, which can be associated with serious harm to the liver, in the Phase 1 clinical trial.

In addition, in our completed Phase 3 clinical trial of ataluren for the treatment of nmCF, five adverse events in the ataluren arm of the trial that involved the renal system led to discontinuation. As compared to the placebo group, the ataluren treatment arm also had a higher incidence of adverse events of creatinine elevations, which can be an indication of impaired kidney function. In the ataluren treatment arm, more severe clinically meaningful creatinine elevations were reported in conjunction with cystic fibrosis pulmonary exacerbations. These creatinine elevations were associated with concomitant treatment with antibiotics associated with impaired kidney functions, such as aminoglycosides or vancomycin. This led to the subsequent prohibition of concomitant use of ataluren and these antibiotics, which was successful in addressing this issue in the clinical trial. If patients in the ataluren arm of a confirmatory Phase 3 clinical trial for the treatment of nmCF exhibit clinically meaningful creatinine elevations, the EMA or the FDA might not approve ataluren for this indication or could require that we instruct physicians to frequently monitor patients for these abnormalities or impose other conditions, which may be an impediment to the use of ataluren because of concerns related to its safety and convenience.

Further, in 2011, we suspended development of our oncology product candidate PTC299, an inhibitor of production of vascular endothelial growth factor, or VEGF, in part because of two cases of severe liver toxicity that occurred in our clinical trials of PTC299 and in part because of our limited resources available at that time.

Our focus on the discovery and development of product candidates that target post-transcriptional control processes is unproven, and we do not know whether we will be able to develop any products of commercial value.

Our scientific approach focuses on the discovery and development of product candidates that target post-transcriptional control processes. While a number of commonly used drugs and a growing body of research validate the importance of post-transcriptional control processes in the origin and progression of a number of diseases, no existing drugs have been specifically designed to alter post-transcriptional control processes in the same manner as ataluren or our other product candidates. As a result, our focus on targeting these processes may not result in the discovery and development of commercially viable drugs

that safely and effectively treat genetic disorders or other diseases. In addition, even if we are successful in developing and receiving regulatory approval for a commercially viable drug that treats an approved indication by targeting a particular post-transcriptional control process, we may not receive regulatory approval for additional indications. Furthermore, we may not receive regulatory approval for product candidates that target different post-transcriptional control processes. If we fail to develop and commercialize viable drugs, we will not achieve commercial success.

Even if ataluren or any other product candidate receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If ataluren or any of our other product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or any profits from operations. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects;
- the ability to offer our product candidates for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement; and
- any restrictions on concomitant use of other medications, such as a restriction that nmCF patients taking ataluren not also use chronic inhaled aminoglycoside antibiotics.

Our ability to negotiate, secure and maintain third-party coverage and reimbursement may be affected by political, economic and regulatory developments in the United States, the European Union and other jurisdictions. Governments continue to impose cost containment measures, and third-party payors are increasingly challenging prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. These and other similar developments could significantly limit the degree of market acceptance of ataluren or any of our other product candidates that receive marketing approval.

***If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be successful in commercializing ataluren or any other product candidate if and when they are approved*.**

We do not have a sales or marketing infrastructure and have no experience in the sale or marketing of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. We plan to establish our own sales and marketing capabilities and promote ataluren in the European Union and the United States with a targeted sales force if and when it is approved. There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales and marketing services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide.

Currently available treatments for Duchenne muscular dystrophy are only palliative. Although there are currently no marketed therapeutics approved to treat the underlying cause of nmDMD, there are other biopharmaceutical companies, including Prosensa Therapeutics and Sarepta Therapeutics, that are developing treatments for Duchenne muscular dystrophy based on a different scientific approach known as exon-skipping. Summit Corporation also has a product candidate in early clinical development designed to increase the production of the protein utrophin, which is functionally similar to dystrophin, to treat Duchenne muscular dystrophy. We believe that ataluren is the only product candidate in clinical trials that is designed to treat the underlying cause of nmDMD by restoring dystrophin activity.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products to manage the symptoms and side effects of cystic fibrosis. These products include Chiron Corporation's TOBI and Genentech, Inc.'s Pulmozyme. Although there are currently no marketed products approved to treat the underlying cause of nmCF, Vertex Pharmaceuticals' CFTR potentiator drug Kalydeco is approved by the FDA as a treatment for cystic fibrosis in patients six years of age and older who have a type of mutation in the CFTR gene known as a gating mutation. Vertex Pharmaceuticals also is developing two other product candidates for the treatment of cystic fibrosis in patients who have a type of mutation in the CFTR gene known as a process block mutation. We believe that ataluren is the only product candidate in clinical trials that is designed to treat the underlying cause of nmCF by restoring CFTR activity.

Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or non-competitive. Our competitors may also obtain marketing approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

We believe that many competitors are attempting to develop therapeutics for the target indications of our product candidates, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs.

Even if we are able to commercialize ataluren or any other product candidate that we develop, the product may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize ataluren or any other product candidate successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the E.U. and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for

ataluren or any other product that we commercialize and, if coverage and reimbursement are available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for ataluren may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We have product liability insurance that covers our clinical trials up to a $5.0 million annual aggregate limit and subject to a per claim deductible. The amount of insurance that we currently hold may not be

adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage when and if we begin commercializing ataluren or any other product candidate that receives marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations currently, and may in the future, involve the use of hazardous and flammable materials, including chemicals and medical and biological materials, and produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and wastes, we cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or disposal of hazardous wastes, we could be held liable for any resulting damages, and any liability could exceed our resources.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We also maintain liability insurance for some of these risks, but our policy excludes pollution and has a coverage limit of $5.0 million.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. For example, we initiated separate Phase 2 clinical trials of ataluren for the treatment of hemophilia in 2009 and the metabolic disorder methylmalomic acidemia in 2010, but then suspended these clinical trials to focus on the development of ataluren for nmDMD and nmCF when we found variability in the assays used in these trials and preliminary data from these trials did not indicate definitive evidence of activity. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

We have based our research and development efforts on small-molecule drugs that target post-transcriptional control processes. Notwithstanding our large investment to date and anticipated future expenditures in proprietary technologies, including GEMS and our alternative splicing technology, which we use in the discovery of these molecules, we have not yet developed, and may never successfully develop, any marketed drugs using this approach. As a result of pursuing the development of product candidates using our proprietary technologies, we may fail to develop product candidates or address indications based on other scientific approaches that may offer greater commercial potential or for which there is a greater

likelihood of success. Research programs to identify new product candidates require substantial technical, financial and human resources. These research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks related to our dependence on third parties

Use of third parties to manufacture our product candidates may increase the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate manufacturing facilities for the production of clinical or commercial supplies of our product candidates. We have limited personnel with experience in drug manufacturing and lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on third parties for supply of the active pharmaceutical ingredients in our product candidates. Our strategy is to outsource all manufacturing of our product candidates and products to third parties.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of any of our product candidates. We obtain our supply of the bulk drug substance for ataluren from a single third-party manufacturer. We engage a separate manufacturer to provide fill and finish services for the finished product that we are using in our clinical trials of ataluren. We may be unable to conclude agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practice, or cGMP, regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

If the third parties that we engage to manufacture product for our preclinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that receive regulatory approval on a timely and competitive basis.

We rely on third parties to conduct our clinical trials and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We do not independently conduct clinical trials for our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to perform this function. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. Similar Good Clinical Practices and transparency requirements apply in the European Union. Failure to comply with such requirements, including with respect to clinical trials conducted outside the European Union, can also lead regulatory authorities to refuse to take into account clinical trial data submitted as part of an MAA.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Our product development costs will increase if we experience delays in testing or obtaining marketing approvals.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We currently depend, and expect to continue to depend, on collaborations with third parties for the development and commercialization of some of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

For each of our product candidates, we plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaborations with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation, for our spinal muscular atrophy program. We generally plan to seek collaborators for the development and commercialization of product candidates that have high anticipated development costs or that are directed at indications for which a potential collaborator has a particular expertise or markets that require a large sales and marketing organization to serve effectively. Our likely collaborators for any marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies.

We will have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In particular, the successful development of a product candidate from our spinal muscular atrophy program will initially depend on the success of our collaborations with the SMA Foundation and Roche and whether Roche pursues clinical development of any compounds identified under our collaboration with the SMA Foundation.

Collaborations involving our product candidates, including our collaborations with the SMA Foundation and Roche, pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;
- we may grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaborators have terminated collaborations with us in the past. For example, in 2008, we entered into a collaboration with Genzyme Corporation for the development and commercialization of ataluren under which we granted to Genzyme rights to commercialize ataluren in all countries other than the United States and Canada. In 2011, we restructured the collaboration and regained worldwide rights to ataluren, with Genzyme obtaining an option to commercialize ataluren in indications other than nmDMD outside the United States and Canada. In 2012, this option expired without being exercised by Genzyme and the collaboration terminated.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish additional collaborations, we may have to alter our development and commercialization plans.

Our product development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate further with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge; and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In

addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

We are a party to a number of license agreements and expect to enter into additional licenses in the future. Our existing licenses impose, and we expect that future licenses will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the right to terminate the license, in which event we might not be able to market any product that is covered by these agreements, which could materially adversely affect the value of the product candidate being developed under such license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms, or cause us to lose rights in important intellectual property or technology.

We have also received grant funding for some of our development programs from philanthropic organizations and patient advocacy groups pursuant to agreements that impose development and commercialization diligence obligations on us. If we fail to comply with these obligations, the applicable organization could require us to grant to the organization exclusive rights under certain of our intellectual property, which could materially adversely affect the value to us of product candidates covered by that intellectual property even if we are entitled to a share of any consideration received by such organization in connection with any subsequent development or commercialization of the product candidates.

Some of our patented technology was developed with U.S. federal government funding. When new technologies are developed with U.S. government funding, the government obtains certain rights in any resulting patents, including a nonexclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise "march-in" rights to use or allow third parties to use our patented technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, U.S. government-funded inventions must be reported to the government and U.S. government funding must be disclosed in any resulting patent applications. Furthermore, our rights in such inventions are subject to government license rights and certain restrictions on manufacturing products outside the United States.

Risks related to our intellectual property

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States and in certain foreign jurisdictions related to our novel technologies and product candidates that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, if we license technology or product candidates from third parties in the future, these license agreements may not permit us to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering this intellectual property. These agreements could also give our licensors the right to enforce the licensed patents without our involvement, or to decide not to enforce the patents at all. Therefore, in these circumstances, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. In addition, we may not pursue or obtain patent protection in all major markets. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Moreover, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office or become involved in opposition, derivation, reexamination, inter partes review, post grant review, interference proceedings or other patent office proceedings or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent

applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. In addition, other companies may attempt to circumvent any regulatory data protection or market exclusivity that we obtain under applicable legislation, which may require us to allocate significant resources to preventing such circumvention. Legal and regulatory developments in the European Union and elsewhere may also result in clinical trial data submitted as part of an MAA becoming publicly available. Such developments could enable other companies to circumvent our intellectual property rights and use our clinical trial data to obtain marketing authorizations in the European Union and in other jurisdictions. Such developments may also require us to allocate significant resources to prevent other companies from circumventing or violating our intellectual property rights. Our attempts to prevent third parties from circumventing our intellectual property and other rights may ultimately be unsuccessful. We may also fail to take the required actions or pay the necessary fees to maintain our patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file claims, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, inter partes review

or post-grant review proceedings before the U.S. Patent and Trademark Office. The risks of being involved in such litigation and proceedings may also increase as our product candidates approach commercialization, and as we gain greater visibility as a public company. Third parties may assert infringement claims against us based on existing or future intellectual property rights. We may not be aware of all such intellectual property rights potentially relating to our product candidates. For example, we have not conducted a recent freedom-to-operate search or analysis for ataluren. Any freedom-to-operate search or analysis previously conducted may not have uncovered all relevant patents and patent applications, and there may be pending or future patent applications that, if issued, would block us from commercializing ataluren. Thus, we do not know with certainty whether ataluren, any other product candidate, or our commercialization thereof, does not and will not infringe any third party's intellectual property.

If we are found to infringe a third party's intellectual property rights, or in order to avoid or settle litigation, we could be required to obtain a license to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us, and could require us to make substantial payments. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

For example, it is possible that one or more third parties might bring a patent infringement or other legal proceeding against us regarding ataluren. We are aware of an issued U.S. patent and international patent applications that purport to disclose or contain claims to chemical scaffolds that are sufficiently broad that they could be read to encompass ataluren, even though neither the issued U.S. patent nor any of the international patent applications specifically discloses ataluren. In order to successfully challenge the validity of any issued U.S. patent, we would need to overcome a presumption of validity. This burden is a high one requiring us to present clear and convincing evidence as to the invalidity of these claims. There is no assurance that a court would find these claims to be invalid. In addition, we believe that our testing of ataluren in clinical trials for the purpose of seeking FDA approval would be a valid defense against any infringement claims in the United States based on the availability of a statutory exemption. However, there can be no assurance that our interpretation of the statutory exemption would be upheld, and the statutory exemption would only cover our preclinical research activities, and not the commercialization of ataluren.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while we typically require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and could distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be obtained or independently developed by a competitor, our competitive position would be harmed.

We have not yet registered our trademarks in all of our potential markets, and failure to secure those registrations could adversely affect our business.

Our trademark applications may not be allowed for registration, and our registered trademarks may not be maintained or enforced. During trademark registration proceedings, we may receive rejections. Although we are given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the U.S. Patent and Trademark Office and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we do not secure registrations for our trademarks, we may encounter more difficulty in enforcing them against third parties than we otherwise would.

Risks related to regulatory approval of our product candidates

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates, including ataluren, and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market ataluren or any of our other product candidates from regulatory authorities in any jurisdiction. In 2011, we submitted a new drug application, or NDA, to the FDA for approval of ataluren for the treatment of nmDMD. The FDA refused to file this NDA on the grounds that the NDA did not contain substantial evidence of effectiveness based on the single placebo controlled Phase 2b clinical trial conducted to date.

We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party contract research organizations to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that ataluren or any of our other product candidates are not effective or only moderately effective, or have undesirable or unintended side effects, toxicities, safety profile or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately

obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

We may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, or can be classified as a similar medicinal product within the meaning of E.U. law, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including Europe and the United States, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan drug designations from the EMA and from the FDA for ataluren for the treatment of nmDMD and nmCF. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the EMA from accepting another marketing application for a similar medicinal product or the FDA from approving another marketing application for the same drug for that time period. The applicable market exclusivity period is ten years in the European Union and seven years in the United States. The E.U. exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is no longer justified.

In the European Union, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. For a drug such as ataluren, which is composed of small molecules, the FDA defines "same drug" as a drug that contains the same active molecule and is intended for the same use. Obtaining orphan drug exclusivity for ataluren for these indications, both in Europe and in the United States, may be important to the product candidate's success. If a competitor obtains orphan drug exclusivity for a product competitive with ataluren before we do and if the competitor's product is the same drug or a similar medicinal product as ours, we could be excluded from the market. Even if we obtain orphan drug exclusivity for ataluren for these indications, we may not be able to maintain it. For example, if a competitive product that is the same drug or a similar medicinal product as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product.

The fast track designation for ataluren may not actually lead to a faster development or regulatory review or approval process.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. We have obtained a fast track designation from the FDA for ataluren for the treatment of both nmDMD and nmCF. However, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw our fast track designation if the FDA believes that the designation is no longer supported by data from our clinical development program. Our fast track designation does not guarantee that we will qualify for or be able to take advantage of the FDA's expedited review procedures.

Any product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any product for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. The FDA's requirements include submissions of safety and other post-marketing information and reports, registration requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and complaints and corresponding maintenance of records and documents, requirements regarding the distribution of samples to healthcare professionals and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval, including the requirement of risk evaluation and mitigation strategy, or REMS. The FDA also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not comply with the laws governing promotion of approved drugs, we may be subject to enforcement action for off-label promotion. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- changes to or restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to implement a REMS;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions; or
- the imposition of civil or criminal penalties.

Non-compliance with E.U. requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Failure to obtain or maintain regulatory approval in international jurisdictions would prevent us from marketing our products abroad.

In order to market and sell ataluren and our other products in the European Union and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

Our ability to obtain and maintain conditional marketing authorizations in the European Union is limited to specific circumstances and subject to several conditions and obligations. Conditional marketing authorizations based on incomplete clinical data may be granted for a limited number of listed medicinal products for human use, including products designated as orphan medicinal products under E.U. law, if (1) the risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) unmet medical needs will be fulfilled and (4) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations, including with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data, may be specified in the conditional marketing authorization. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions. Even if we obtain conditional approval for ataluren for the treatment of either or both of nmDMD and nmCF, we may not be able to renew such conditional approval. A failure to renew any conditional approval that we obtain prior to full approval for the applicable indication would prevent us from continuing to market ataluren for such indication.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the member states of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various E.U. member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we may be required to conduct a clinical trial or other studies that compare the

cost-effectiveness of our product candidate to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be adversely affected.

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates, including ataluren, for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- The federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federally funded healthcare programs such as Medicare and Medicaid. This statute has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others.

- The federal False Claims Act imposes civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. The government and *qui tam* relators have brought False Claims Act actions against pharmaceutical companies on the theory that their practices have caused false claims to be submitted to the government. There is also a separate false claims provision imposing criminal penalties.

- The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

- HIPAA also imposes criminal liability for knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services.

- The federal Physician Sunshine Act requirements under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, referred to together as the Affordable Care Act, require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value made to or at the request of covered recipients, such as physicians and teaching hospitals, and physician ownership and investment interests in such manufacturers. Payments made to physicians and research institutions for clinical trials are included within the ambit of this law.

- Analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. Exclusion, suspension and debarment from government funded healthcare programs would significant impact our ability to commercialize, sell or distribute any drug. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of ataluren or any of our other product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates, including ataluren, for which we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

More recently, in March 2010, President Obama signed into law the Affordable Care Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Effective October 1, 2010, the Affordable Care Act revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which

may affect our business practices with health care practitioners. We will not know the full effects of the Affordable Care Act until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the full effect of the Affordable Care Act, the new law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our products. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs.

Risks related to employee matters and managing growth

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Dr. Stuart W. Peltz, our co-founder and Chief Executive Officer, and the other principal members of our executive and scientific teams. Although we have formal employment agreements with each of our executive officers, these agreements do not prevent our executives from terminating their employment with us at any time. We do not maintain "key person" insurance on any of our executive officers. The loss of the services of any of these persons might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks related to our common stock and this offering

After this offering, our executive officers, directors and principal stockholders will maintain the ability to control all matters submitted to stockholders for approval.

Upon the closing of this offering, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately % of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws that will become effective upon the closing of this offering may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- provide for a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of

the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

We expect the initial public offering price of our common stock to be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent outstanding options or warrants are exercised, you will incur further dilution. Based on an assumed initial public offering price of $ per share, which is the midpoint of the price range listed on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering and the assumed initial public offering price. In addition, purchasers of common stock in this offering will have contributed approximately % of the aggregate price paid by all purchasers of our stock but will own only approximately % of our common stock outstanding after this offering.

An active trading market for our common stock may not develop.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters. Although we have applied to list our common stock on the NASDAQ Global Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares or at all.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.

Our stock price may be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of ataluren and any other product candidate that we develop;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk factors" section.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- providing only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in this prospectus. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act also provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to delay such adoption of new or revised accounting standards, and as a result, we may not comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for public companies that are not emerging growth companies. As a result of such election, our financial statements may not be comparable to the financial statements of other public companies.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Act, the listing requirements of the NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

However, for as long as we remain an emerging growth company, we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies as described in the preceding risk factor. We may remain an emerging growth company until the end of the fiscal year in which the fifth anniversary of this offering occurs, although if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have annual gross revenues of $1 billion or more in any fiscal year, we would cease to be an emerging growth company as of December 31 of the applicable year. We also would cease to be an emerging growth company if we issue more than $1 billion of non-convertible debt over a three-year period.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, as an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm until we are no longer an emerging growth company. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Because we do not anticipate paying any cash dividends on our capital in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the development and growth of our business. In addition, the terms of existing or any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding shares of common stock based on the number of shares outstanding as of , 2013. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. Of the remaining shares, shares are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold after the offering as described in the "Shares eligible for future sale" section of this prospectus. Moreover, after this offering, holders of an aggregate of shares of our common stock will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus.

Special note regarding forward-looking statements

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus include, among other things, statements about:

- the timing and conduct of our clinical trials of ataluren for the treatment of Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations, including statements regarding the timing of initiation and completion of the trials and the period during which the results of the trials will become available;
- the timing of and our ability to obtain marketing approval, including conditional approval in the European Union, of ataluren and our other product candidates, and the ability of ataluren and our other product candidates to meet existing or future regulatory standards;
- the potential receipt of revenues from future sales of ataluren;
- our plans to pursue development of ataluren for additional indications other than Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations;
- our plans to pursue research and development of other product candidates;
- the potential advantages of ataluren;
- the rate and degree of market acceptance and clinical utility of ataluren;
- our estimates regarding the potential market opportunity for ataluren;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for manufacture of ataluren and our other product candidates;
- our intellectual property position;
- our expectations related to the use of proceeds from this offering;
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the impact of government laws and regulations; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking

statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the "Risk factors" section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this prospectus are made as of the date of this prospectus, and we do not assume any obligation to update any forward-looking statements.

Use of proceeds

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $ million, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise in full their over-allotment option, we estimate that the net proceeds from this offering will be approximately $ million.

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions.

As of , we had cash and cash equivalents of approximately $ million. We currently estimate that we will use the net proceeds from this offering, together with our cash and cash equivalents, as follows:

- approximately $ million to fund the clinical development of and seek marketing approval for ataluren for the treatment of nmDMD;
- approximately $ million to fund the clinical development of and seek marketing approval for ataluren for the treatment of nmCF;
- approximately $ million to pursue the development of ataluren for additional indications;
- approximately $ million to fund our other research and development programs; and
- the remainder for working capital and other general corporate purposes.

This expected use of the net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and commercialization efforts, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our product candidates, and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering. We have no current agreements, commitments or understandings for any material acquisitions or licenses of any products, businesses or technologies.

Based on our planned use of the net proceeds from this offering and our existing cash and cash equivalents described above, we estimate that such funds will be sufficient to enable us to . We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. This estimate assumes, among other things, that we do not receive conditional approval to market ataluren for nmDMD or nmCF prior to completing a Phase 3 clinical trial for the applicable indication and, as a result, that we do not incur significant related commercialization expenses prior to such time.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

Dividend policy

We have never declared or paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay cash dividends to holders of our common stock in the foreseeable future. Additionally, our ability to pay dividends on our common stock is limited by restrictions under the terms of the agreements governing our debt facility.

Capitalization

The following table sets forth our cash and cash equivalents and capitalization as of December 31, 2012:

- on an actual basis;
- on a pro forma basis to give effect to:
    - the reclassification of our previously outstanding preferred stock into our series five junior preferred stock, the reverse stock split of our common stock and the issuance of an aggregate of 4,999,954 shares of our series four senior preferred stock, including upon the conversion of convertible promissory notes, for aggregate proceeds of approximately $60 million, on March 7, 2013; and
    - the automatic conversion of all outstanding shares of our preferred stock, including shares of our series four senior preferred stock that we issued on March 7, 2013, into an aggregate of 13,795,956 shares of our common stock upon the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Our capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this table together with our financial statements and the related notes appearing at the end of this prospectus and the "Management's discussion and analysis of financial condition and results of operations" section of this prospectus.

As of December 31, 2012	Actual	Pro forma	Pro forma as adjusted
Cash and cash equivalents	$ 2,725,702		
Series one convertible preferred stock, $0.001 par value per share, designated 2,000,000 shares; issued and outstanding 1,483,337 shares at December 31, 2012	62,263,852		
Series two convertible preferred stock, $0.001 par value per share, designated 13,750,000 shares; issued and outstanding 10,701,405 shares at December 31, 2012	18,182,129		
Series three convertible preferred stock, $0.001 par value per share, designated 13,750,000 shares; issued and outstanding 2,853,517 shares at December 31, 2012	377,787		
Debt obligations	4,882,981		
Stockholders' deficit:			
Common stock, $0.001 par value per share, authorized 26,000,000 shares; issued and outstanding 545,345 shares at December 31, 2012	545		
Additional paid-in capital	177,583,672		
Accumulated deficit	(277,224,718)		
Total capitalization (deficit)	(13,933,752)		

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) each of cash, cash equivalents and short-term investments, additional paid-in capital, total stockholders' equity and total capitalization on a pro forma as adjusted basis by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions.

The table above does not include:

- shares of our common stock issuable upon the exercise of stock options outstanding as of , at a weighted-average exercise price of $ per share;
- additional shares of our common stock available for future issuance as of under our 2009 equity and long term incentive plan and our 2013 stock incentive plan;
- additional shares of our common stock that will be available for future issuance, as of the closing of this offering, under our 2013 public company stock incentive plan; and
- shares of our common stock issuable upon the exercise of warrants outstanding as of , at a weighted-average exercise price of $ per share.

Dilution

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the public offering price per share of our common stock and the pro forma net tangible book value per share of our common stock after this offering.

Our historical net tangible book value as of was $ million, or $ per share of our common stock. Historical net tangible book value per share represents the amount of our total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding.

Our pro forma net tangible book value as of was $ million, or $ per share of our common stock. Pro forma net tangible book value per share represents the amount of our total tangible assets less our total liabilities, divided by the pro forma number of shares of our common stock outstanding after giving effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 13,795,956 shares of our common stock upon the closing of this offering.

After giving effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma net tangible book value as of would have been $ million, or $ per share. This represents an immediate increase in pro forma net tangible book value per share of $ to existing stockholders and immediate dilution of $ in pro forma net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share		$
Historical net tangible book value per share as of	$	
Increase per share attributable to the conversion of outstanding preferred stock and reclassification of preferred stock warrants		
Pro forma net tangible book value per share as of		
Increase in net tangible book value per share attributable to new investors		
Pro forma net tangible book value per share after this offering		
Dilution per share to new investors		$

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma net tangible book value by approximately $ or our pro forma net tangible book value per share by approximately $, and dilution per share to new investors by approximately $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their over-allotment option or if any additional shares are issued in connection with outstanding options or warrants, you will experience further dilution.

The following table summarizes, on a pro forma basis as of , the total number of shares purchased from us, the total consideration paid, or to be paid, and the average price per share paid, or to

be paid, by existing stockholders and by new investors in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing shares in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	Shares purchased		Total consideration		Average price per share
	Number	Percent	Amount	Percent	
Existing stockholders		%	$	%	$
New investors					
Total		100%		100%	

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by $ million and increase (decrease) the percentage of total consideration paid by new investors by approximately %, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same.

The table above is based on actual shares of our common stock outstanding as of and additional shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering.

The table above does not include:

- shares of our common stock issuable upon the exercise of stock options outstanding as of , at a weighted-average exercise price of $ per share;
- additional shares of our common stock available for future issuance as of under our 2009 equity and long term incentive plan;
- additional shares of our common stock that will be available for future issuance, as of the closing of this offering, under our 2013 public company stock incentive plan; and
- shares of our common stock issuable upon the exercise of warrants outstanding as of , at a weighted-average exercise price of $ per share.

If the underwriters exercise in full their over-allotment option, the following will occur:

- the percentage of shares of our common stock held by existing stockholders will decrease to approximately % of the total number of shares of our common stock outstanding after this offering; and
- the number of shares of our common stock held by new investors will increase to , or approximately % of the total number of shares of our common stock outstanding after this offering.

Selected financial data

You should read the following selected financial data together with our financial statements and the related notes appearing at the end of this prospectus and the "Management's discussion and analysis of financial condition and results of operations" section of this prospectus. We have derived the consolidated statements of operations data for the years ended December 31, 2011 and 2012 and the consolidated balance sheet data as of December 31, 2011 and 2012 from our audited financial statements included in this prospectus. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our results for any interim period are not necessarily indicative of results to be expected for a full fiscal year.

	Year ended December 31,	
Statement of operations data	**2011**	**2012**
	(in thousands, except share and per share data)	
Revenues:		
Collaboration revenue	$ 98,961	$ 28,779
Grant revenue	6,451	5,167
Total revenues	105,412	33,946
Operating expenses:		
Research and development	58,677	46,139
General and administrative	16,153	14,615
Total operating expenses	74,830	60,754
Income (loss) from operations	30,582	(26,808)
Interest expense, net	(2,444)	(1,210)
Other income, net	461	1,783
Income (loss) before tax benefit	28,599	(26,235)
Income tax benefit	2,306	—
Net income (loss)	30,905	(26,235)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	159,954
Less beneficial conversion charge	—	(378)
Net income attributable to common stockholders	$ 30,905	$ 133,341
Net income per share(1)		
Basic	$ 23.95	$ 219.76
Diluted	$ 4.55	$ 42.50
Weighted-average shares outstanding:		
Basic	1,089	3,328
Diluted	5,729	17,205

(1) See Note 8 to our financial statements appearing at the end of this prospectus regarding the calculation of net income per share.

Balance sheet data	As of December 31, 2011	2012
	(in thousands)	
Cash, cash equivalents and short-term investments	$ 28,431	$ 2,726
Working capital (deficit)	(10,091)	(23,564)
Total assets	44,148	13,072
Long-term debt, including current portion	11,689	4,883
Convertible preferred stock	214,380	80,824
Accumulated deficit	(250,612)	(277,225)
Total stockholders' deficit	(238,605)	(99,641)

Management's discussion and analysis of financial condition and results of operations

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and the related notes appearing at the end of this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the "Risk factors" section of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes. Our lead product candidate is ataluren for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. In addition to ataluren, we have a pipeline of product candidates that are in preclinical development. Our preclinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including neuromuscular disease, oncology and infectious disease.

We are currently initiating a Phase 3 clinical trial of ataluren for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD. We expect to dose the first patient in this trial in the first half of 2013. In October 2012, we submitted a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, for conditional approval of ataluren for the treatment of nmDMD. We plan to complete our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD before applying for marketing approval from the U.S. Food and Drug Administration, or FDA. We are also planning a Phase 3 clinical trial of ataluren for the treatment of cystic fibrosis caused by nonsense mutations, or nmCF. Subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design, we plan to pursue conditional approval of ataluren in the European Union for the treatment of nmCF and, in the second half of 2013, to initiate the confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. Our goal in these continuing discussions is to achieve consensus between the EMA and the FDA that a single placebo controlled Phase 3 clinical trial can serve as the basis for full approval of ataluren to treat nmCF in both the European Union and the United States.

To date, we have financed our operations primarily through private placements of our preferred stock, collaborations, bank debt and convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. As of December 31, 2012, we had an accumulated deficit of $277.2 million. We had net income of $30.9 million for the year ended December 31, 2011, including a $79 million revenue adjustment due to the termination of a collaboration with Genzyme Corporation, or Genzyme, and a net loss of $26.2 million for the year ended December 31, 2012.

We anticipate that our expenses will increase substantially in connection with initiating and continuing confirmatory Phase 3 clinical trials for ataluren for the treatment of nmDMD and nmCF and seeking marketing approval for ataluren for these indications in the European Union and the United States. If we obtain marketing approval of ataluren for either nmDMD or nmCF, we also expect to incur significant sales,

marketing, distribution and manufacturing expenses, as well as ongoing research and development expenses for our other product candidates. The timing of commercialization expenses for ataluren depends in part on whether we receive conditional approval for ataluren for either or both of nmDMD and nmCF. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Although we cannot reasonably estimate the amount of these additional public company costs, we expect that these costs will include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts. We will need to generate significant revenues to achieve and sustain profitability, and we may never do so.

Financial operations overview

Revenues

To date, we have not generated any product sale revenues. Based on our current plans, we do not expect to generate significant product revenues unless and until we obtain marketing approval for, and commercialize, ataluren for the treatment of nmDMD or nmCF. The timing of any product revenues depends in part on whether we receive conditional approval for ataluren for either or both of nmDMD and nmCF. Our revenues to date have consisted of collaborative agreements revenues and grant revenues. We had revenues of $105.4 million for the year ended December 31, 2011, including a $79 million revenue adjustment due to the modification of a collaboration with Genzyme in 2011, and $33.9 million for the year ended December 31, 2012.

We have ongoing collaborations with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation, for our spinal muscular atrophy program and early stage discovery arrangements with other institutions. During 2011, our collaboration with Genzyme was modified and later terminated.

Genzyme. In July 2008, we entered into an exclusive global collaboration with Genzyme to develop and commercialize ataluren for the treatment of genetic disorders due to nonsense mutations. Under the terms of this agreement, we granted Genzyme rights to commercialize ataluren in all countries except the United States and Canada, which rights we retained. Genzyme made a nonrefundable, upfront payment to us of $100,000,000 in July 2008, which we then began recognizing over the estimated period of performance under the arrangement.

In August 2011, we announced a restructuring of the agreement with Genzyme. Under the terms of the restructuring, we regained worldwide rights to ataluren and Genzyme made an additional payment to us in exchange for an option to commercialize ataluren in indications other than nmDMD outside the United States and Canada. In March 2012, Genzyme declined to exercise the option, the option expired and the collaboration terminated.

We evaluated the August 2011 restructuring of the agreement and determined it to be a material modification to the original agreement for financial reporting purposes pursuant to the revised multiple-element revenue recognition guidance. We reevaluated the collaboration arrangement under this revised guidance and recorded a one-time adjustment to our deferred revenue balance to reflect the value of the remaining performance obligations under the restructured agreement as represented by the best estimate

of selling price. As a result of this reevaluation, we recognized approximately $79 million of existing deferred revenue as of the modification date.

Roche and the SMA Foundation. In November 2011, we entered into a license and collaboration agreement with Roche and the SMA Foundation pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our spinal muscular atrophy sponsored research program with the SMA Foundation, as described below, and to research, develop and commercialize other small molecule compounds with potential for therapeutic use in patients with spinal muscular atrophy. Pursuant to the license and collaboration agreement, Roche paid us an upfront non-refundable payment of $30.0 million.

Grant revenue. We receive grant funding from various institutions and governmental bodies. The grants are typically for early discovery research, and generally the grant program lasts from two to five years.

Research and development expense

Research and development expenses consist of the costs associated with our research activities, as well as the costs associated with our drug discovery efforts, conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings. Our research and development expenses consist of:

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;
- employee-related expenses, which include salaries and benefits, including stock-based compensation, for the personnel involved in our drug discovery and development activities; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We use our employee and infrastructure resources across multiple research projects, including our drug development programs. We track expenses related to our clinical programs and certain preclinical programs on a per project basis.

We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we initiate and continue confirmatory Phase 3 clinical trials of ataluren for the treatment of nmDMD and nmCF, continue our research activities in our preclinical programs and initiate clinical development of other product candidates. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs and product candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs.

	Year ended December 31,	
	2011	2012
	(in thousands)	
Ataluren	$ 23,471	$ 15,700
Antibacterial	834	1,989
BMI1	2,422	2,254
Spinal muscular atrophy	3,718	1,664
Other research and preclinical	28,232	24,532
Total research and development	$ 58,677	$ 46,139

We expect that our total costs for the confirmatory Phase 3 clinical trial of ataluren for nmDMD will be approximately $31 million and for nmCF will be approximately $38 million.

The successful development of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our clinical trials and other research and development activities;
- the potential benefits of our product candidate over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our product candidates that we are developing or may develop in the future;
- clinical trial results;
- the terms and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of ataluren or any other product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of ataluren or any other product candidate or if we experience significant delays in enrollment in any our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and administrative expense

General and administrative expense consists primarily of salaries and other related costs for personnel, including stock-based compensation expenses, in our executive, legal, business development, finance, accounting, information technology and human resource functions. Other general and administrative expenses include facility-related costs not otherwise included in research and development expense; advertising and promotional expenses; costs associated with industry and trade shows; and professional fees for legal services, including patent-related expenses, and accounting services.

We expect that general and administrative expense will increase in 2013 and in future periods as a result of increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses associated with being a public company and costs incurred to seek collaborations with respect to any of our product candidates, among other factors.

Interest expense, net

Interest expense, net consists of interest related to our secured debt facility.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in the notes to our financial statements appearing at the end of this prospectus, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Revenue recognition

We recognize revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

Our revenue is generated primarily through collaborative research and development and licensing agreements and grants.

The terms of these agreements typically include payments of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding; and royalties on future product sales. In addition, we generate service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

For existing collaborations entered into prior to the adoption in 2011 of the revised multiple element revenue recognition guidance described below, we recognize revenue consistent with the approach established at the inception of each arrangement. For these existing collaborations, where we have continued involvement, we recorded nonrefundable, upfront fees as deferred revenue and recognize revenue on a straight line basis as collaboration revenue over the expected performance period.

For new collaborations or for material modifications made to existing collaborations, in 2011, we adopted the updated multiple element revenue recognition guidance. Under this new guidance, all non-contingent arrangement consideration is allocated to the identified units of accounting based on their relative selling price at inception of the collaboration arrangement. We derive the selling price using a combination of internal subjective and available external objective information, such as comparable transactions. We recognize revenue commensurate with delivery, such as in the case with delivery of a license, or ratably over the course of a service period, as appropriate, such as in the case of ongoing research and development activities.

We evaluate all contingent consideration earned, such as a milestone payment, using the criteria as provided by the Financial Accounting Standards Board, or FASB, guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, we evaluate if milestone payments are substantive. The criteria requires that (1) we determine if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved. We recognize royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

We recognize reimbursements for research and development costs under collaboration agreements as revenue as the services are performed. We record these reimbursements as revenue and not as a reduction of research and development expenses as we have the risks and rewards as the principal in the research and development activities.

Our principal obligation under our grant agreements is to conduct the internal or external research in the specific field funded by the grant. We determine, through the grant's normal research process, which research and development projects to pursue. We recognize grant revenues as the research activities are performed. If the grant includes an upfront payment, we defer the amount and recognize it as revenue as the expenditures are incurred.

Accrued expenses

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us. Examples of estimated accrued expenses include:

- fees paid to contract research organizations in connection with preclinical and toxicology studies and clinical trials;
- fees paid to investigative sites in connection with clinical trials;
- fees paid to contract manufacturers in connection with the production of clinical trial materials; and
- professional service fees.

Share-based compensation

We expect to grant additional stock options that will result in additional share-based compensation expense. Accordingly, we describe below the methodology we have employed to date in measuring such expenses. Following the consummation of this offering, stock option values will be determined based on the market price of our common stock.

We utilized various valuation methodologies in accordance with the framework of the 2004 American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity*

Securities Issued as Compensation, or the AICPA Practice Aid, to estimate the fair value of our common stock. The methodologies included an option pricing method to estimate our underlying equity value, and a methodology that determined an estimated value under an initial public offering, or IPO, scenario and a sale scenario based upon an assessment of the probability of occurrence of each scenario. Each valuation methodology includes estimates and assumptions that require judgment. These estimates include assumptions regarding future performance, including the completion of clinical trials and the time to complete an IPO or sale of the company. As with any valuation, significant changes to the key assumptions used in the valuations could result in different fair values of common stock at each valuation date. Factors that we considered in determining the fair value of our common stock include:

- pricing of private sales of our preferred stock;
- prior valuations of stock grants and preferred stock sales and the effect of events, including the progression of our product candidates, that have occurred between the time of the grants or sales;
- comparative rights and preferences of the security being granted compared to the rights and preferences of our other outstanding equity;
- comparative values of public companies discounted for the risk and limited liquidity provided for in the shares we are issuing;
- estimates and analysis provided by management and contemporaneous valuations;
- perspective provided by investment banks, including the likelihood of an initial public offering and our potential value in an initial public offering; and
- general economic trends and external market conditions affecting the biopharmaceutical industry.

We measure the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the entire award.

The fair value of options is calculated using the Black-Scholes option pricing model to determine the fair value of stock options on the date of grant based on key assumptions such as stock price, expected volatility and expected term. Our estimates of these assumptions are primarily based on contemporaneous valuations, historical data, peer company data and judgment regarding future trends and factors. This is a distinct valuation process from that used to determine the fair value of our common stock for purposes of establishing the exercise price of stock options that we grant.

The fair value of grants made in the years ended December 31, 2011 and 2012 was contemporaneously estimated on the date of grant using the following assumptions:

	2011	2012
Risk-free interest rate	2.40%	1.135%
Expected volatility	87%	87%
Expected term	6.00-6.25 years	6.00-6.25 years

We assumed no expected dividends for all grants. The weighted average grant date fair value was $364.80 for options granted during the year ended December 31, 2011 and $160.80 for options granted during the year ended December 31, 2012.

We use the "simplified method" to determine the expected term of options. Under this method, the expected term represents the average of the vesting period and the contractual term. The expected volatility of share options was estimated based on a historical volatility analysis of peers that were similar to us with respect to industry, stage of life cycle, size and financial leverage. The risk-free rate of the options is based on U.S. Government Securities Treasury Constant Maturities yields at the date of grant for a term similar to the expected term of the option.

We recognized share-based compensation expense of approximately $2.8 million during the year ended December 31, 2011 and $2.3 million during the year ended December 31, 2012.

We had total unrecognized compensation cost related to unvested share-based compensation arrangements of $4.0 million as of December 31, 2011 and $2.2 million as of December 31, 2012. We expect to recognize this cost as compensation expense over the weighted average remaining service period of approximately 2.14 years.

The following table sets forth information regarding stock options granted during the years ended December 31, 2011 and 2012:

Grant date	Number of options	Exercise price per share	Common stock fair value per share on grant date	Black-Scholes fair value per share of options
4/27/2011	8,123	$ 490.80	$ 490.80	$ 364.80
1/10/2012	5,715	$ 218.40	$ 218.40	$ 160.80

Stock option grants made on April 27, 2011. Our board of directors granted options to purchase 8,123 shares of common stock on April 27, 2011, with each option having an exercise price of $490.80 per share. In determining this exercise price, our board of directors considered input from management and a valuation of our common stock. We determined the value of our common stock based on the probability weighted expected return method, or PWERM, described in the AICPA Practice Aid. We considered but did not use the market approach because our early stage of development and the absence of clinical trial data from our lead candidate made comparisons to public companies difficult. Similarly, we did not use the income approach because of the uncertain outcomes of our ongoing and future clinical trials.

Under a PWERM analysis, the value of a company's common stock is estimated based upon an analysis of current and future enterprise values, assuming three possible liquidity scenarios: an IPO, a recapitalization of the company and a sale of the company. We considered two significant value inflection points related to the ataluren program. The first inflection point was related to anticipated FDA action regarding our dispute of the FDA's refusal to file our new drug application, or NDA, that we submitted for ataluren for the treatment of nmDMD. The second inflection point was related to anticipated Phase 3 clinical trial results for ataluren for the treatment of nmCF.

After considering the various potential liquidity scenarios for our company and their likely timing, we used a pre-money enterprise value assigned to each scenario based on recent trends in capital markets. To determine the price per share of our common stock, we divided the resulting enterprise value for each liquidity scenario by the number of common shares that would be outstanding under each scenario. The common stock price for each scenario was then assigned a probability based on management's estimates. The resulting probability-weighted common share values were then discounted to present value at a rate that reflected general industry risks. The result was a value of our common stock on a minority, non-marketable basis of $490.80 per share.

Stock option grants made on January 10, 2012. Our board of directors granted options to purchase 5,715 shares of common stock on January 10, 2012, with each option having an exercise price of $218.40 per share. In determining this exercise price, our board of directors considered input from management and a valuation of our common stock. We determined the value of our common stock based on a PWERM analysis as described in the AICPA Practice Aid. We again considered but did not use the market approach because our early stage of development and the absence of clinical trial data from our lead candidate made comparisons to public companies difficult. Similarly, we did not use the income approach because of the uncertain outcomes of our ongoing and future clinical trials.

We considered two significant value inflection points related to the ataluren program. The first inflection point was related to anticipated Phase 3 clinical trial results for ataluren for the treatment of nmCF. The second inflection point was related to U.S. or E.U. regulatory approval of ataluren for the treatment of nmDMD.

After considering the various potential liquidity scenarios for our company and their likely timing, we used a pre-money enterprise value assigned to each scenario based on recent trends in capital markets. To determine the price per share of our common stock, we divided the resulting enterprise value for each liquidity scenario by the number of common shares that would be outstanding under each scenario. The common stock price for each scenario was then assigned a probability based on management's estimates. The resulting probability-weighted common share values were then discounted to present value at a rate that reflected general industry risks. The result was a value of our common stock on a minority, non-marketable basis of $218.40 per share.

The reduction in the value of our common stock from $490.80 to $218.40 between April 2011 and January 2012 primarily reflected the FDA's reaffirmation of its earlier decision to refuse to file our NDA for ataluren for the treatment of nmDMD and the unavailability of results of our Phase 3 clinical trial of ataluren for nmCF by January 2012. Acceptance of our NDA for filing by the FDA could have led to approval of ataluren and a subsequent higher value with respect to the IPO scenario. Given the FDA's reaffirmation of its earlier decision to refuse to file our NDA, the high value IPO scenarios were no longer realistic, resulting in a decrease in the value of our common stock.

Warrant liability

We classify as liabilities warrants to purchase our common stock with nonstandard antidilution provisions and warrants to purchase our preferred stock that include a put feature, regardless of the probability or likelihood that may conditionally obligate us to ultimately transfer assets, and record the estimated fair value of these warrants at each reporting period. We record as gain or loss any change in fair value of these warrants each reporting period in other income on our statement of operations.

Income taxes

As part of the process of preparing our financial statements, we are required to estimate our income taxes in each of the jurisdictions in which we operate. This process involves estimating our actual current tax expense together with assessing temporary differences resulting from differing treatments of items for tax and accounting purposes. These differences result in deferred tax assets and liabilities. As of December 31, 2012, we had federal net operating loss carryforwards of $211.0 million, which expire starting in 2021, and federal research and development credit carryforwards of $5.4 million, which expire starting in 2013. We also had state net operating loss carryforwards of $128.3 million, which expire starting in 2029, and state research and development credit carryforwards of $1.5 million, which expire starting in 2022. The Internal Revenue Code contains provisions that may limit the net operating loss and credit carryforwards available to be used in any given year given certain historical changes in the ownership interests of significant

stockholders. At December 31, 2012, we recorded a full valuation allowance against our net deferred tax asset of approximately $106.9 million, as our management believes it cannot at this time conclude that it is more likely than not they will be realized. If we determine in the future that we will be able to realize all or a portion of our net deferred tax asset, an adjustment to the deferred tax valuation allowance would increase net income in the period in which we make such a determination.

Results of operations

Year ended December 31, 2011 compared to year ended December 31, 2012

(in thousands)	2011	2012	Change 2012 vs. 2011
Revenue	$ 105,412	$ 33,946	$ (71,466)
Research and development expenses	58,677	46,139	(12,538)
General and administrative expenses	16,153	14,615	(1,538)
Interest expense	2,444	1,210	(1,234)
Other income, net	461	1,783	1,322
Tax benefit	2,306	—	(2,306)

Years ended December 31, 2011 and 2012

Revenues. Revenues were $33.9 million in 2012, a decrease of $71.5 million from revenues of $105.4 million in 2011. Collaboration revenue was $28.8 million in 2012, a decrease of $70.2 million from collaboration revenues of $99.0 million in 2011. The decrease resulted primarily from a one-time non cash adjustment in 2011 to our deferred revenue balance to reflect the value of the remaining performance obligations under our restructured agreement with Genzyme. We recognized approximately $79 million of existing deferred revenue under our agreement with Genzyme as of the modification date. Grant revenue was $5.2 million in 2012, a decrease of $1.3 million from grant revenue of $6.5 million in 2011.

Research and development expense. Research and development expense was $46.1 million in 2012, a decrease of $12.6 million, or 21%, from $58.7 million in 2011. The decrease resulted primarily from decreased costs for clinical trials of $5.8 million, decrease in manufacturing of clinical trial supplies of $2.3 million and a decrease in personnel costs of $2.3 million as a result of a reduction in force that we implemented in the second quarter of 2012. Clinical trial expense for 2011 reflected costs associated with our Phase 3 clinical trial of ataluren for the treatment of nmCF, which concluded in November 2011, and a related extension trial and a Phase 3 continuation trial for ataluren for the treatment of nmDMD. Clinical trial expense for 2012 reflected costs associated with the ongoing extension trial for patients who had participated in our Phase 3 clinical trial of ataluren for the treatment of nmCF, the ongoing continuation trial for ataluren for the treatment of nmDMD and a second Phase 3 continuation trial that we initiated in 2012 for ataluren for the treatment of nmDMD.

General and administrative expense. General and administrative expense was $14.6 million in 2012, a decrease of $1.6 million, or 9.5%, from $16.2 million in 2011. The decrease was due principally to decreased personnel costs of $1.6 million as a result of a reduction in force that we implemented in the second quarter of 2012.

Interest expense. Interest expense was $1.2 million in 2012, a decrease of $1.2 million from $2.4 million in 2011. The increase was due to a smaller loan balance in 2012 as we continued to repay outstanding debt.

Other income, net. Other Income, net was $1.8 million in 2012, an increase of $1.3 million from $0.5 million in 2011. The increase was due to the change in fair value related to our warrant liability.

Tax benefit. We recognized a tax benefit related to our sale of net operating losses in the New Jersey Technology Business Tax Certificate Transfer Program. In 2011, our benefit was $2.3 million. We did not qualify for this program in 2012.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses. To date, we have not generated any product sale revenues. We have financed our operations primarily through private placements of our preferred stock, collaborations, bank debt and convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates.

Cash flows

As of December 31, 2012, we had cash and cash equivalents $2.7 million. On March 7, 2013, we closed a private placement of a new series of convertible preferred stock, pursuant to which we issued an aggregate of 4,999,954 shares of our series four senior preferred stock, including upon the conversion of convertible promissory notes, for aggregate proceeds of approximately $60 million.

The following table provides information regarding our cash flows and our capital expenditures for the years ended December 31, 2011 and 2012.

(in thousands)	2011	2012
Cash provided by (used in):		
Operating activities	$ (20,767)	$ (47,928)
Investing activities	27,703	(189)
Financing activities	(7,180)	22,411

Net cash used in operating activities was $20.8 million for the year ended December 31, 2011 and $47.9 million for the year ended December 31, 2012. The net cash used in 2011 and 2012 primarily reflects changes in deferred revenue, including an upfront cash payment of $30 million in 2011 related to the collaboration agreement with Roche for a spinal muscular atrophy program, which is being amortized over the research term, and decreased spending in 2012 on research and development costs due to the completion of our Phase 2b clinical trial of ataluren for nmDMD and our Phase 3 clinical trial of ataluren for nmCF.

Net cash provided by investing activities was $27.7 million for the year ended December 31, 2011. Net cash used in investing activities was $0.2 million for the year ended December 31, 2012. Cash provided by or used in investing activities in 2011 was primarily related to net maturities of investments, and to a lesser extent, purchases of property and equipment.

Net cash used in financing activities was $7.2 million for the year ended December 31, 2011. Net cash used in financing activities in 2011 was attributable to payments on debt obligations. Net cash provided by financing activities was $22.4 million for the year ended December 31, 2012. Net cash provided by financing activities in 2012 was primarily attributable to the $29.3 million in proceeds that we received from a preferred stock financing. Partially offsetting these proceeds were payments on debt obligations of $6.9 million in 2012.

Funding requirements

We anticipate that our expenses will increase substantially in connection with initiating and continuing confirmatory Phase 3 clinical trials for ataluren for the treatment of nmDMD and nmCF and seeking marketing approval for ataluren for these indications in the European Union and the United States. If we

obtain marketing approval of ataluren for either nmDMD or nmCF, we also expect to incur significant selling, marketing, distribution and manufacturing expenses. The timing of commercialization expenses for ataluren depends in part on whether we receive conditional approval for ataluren for either or both of nmDMD and nmCF.

In addition, our expenses will increase if and as we:

- initiate or continue the research and development of ataluren for additional indications and of our other product candidates;
- seek to discover and develop additional product candidates;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, short-term investments and research funding that we expect to receive under our collaborations, will be sufficient to enable us to fund our operating expenses, debt service obligations and capital expenditure requirements until . We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. This estimate assumes, among other things, that we do not receive conditional approval to market ataluren for nmDMD or nmCF in the European Union prior to completing a confirmatory Phase 3 trial for the applicable indication and, as a result, that we do not incur significant related commercialization expenses prior to such time. Our future capital requirements will depend on many factors, including:

- the progress and results of confirmatory Phase 3 clinical trials of ataluren for nmDMD and nmCF;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for ataluren for additional indications and for our other product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs, timing and outcome of regulatory review of ataluren and our other product candidates;
- the costs and timing of commercialization activities, including product sales, marketing, distribution and manufacturing, for any of our product candidates that receive marketing approval;
- subject to receipt of marketing approval, revenue received from commercial sales of ataluren or any of our other product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products and technologies; and
- our ability to establish and maintain collaborations.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates and marketing, distribution or licensing

arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual obligations

The following table summarizes our significant contractual obligations and commercial commitments as of December 31, 2012.

(in thousands)	Total	Less than 1 year	1-3 years	4-5 years	More than 5 years
Debt obligations	$ 4,883	$4,444	$ 439	$ —	$ —
Operating and equipment lease obligations(1)	5,388	879	2,546	1,801	162
Total fixed contractual obligations	$ 10,271	$5,323	$ 2,985	$ 1,801	$ 162

(1) We lease office space under a noncancelable operating lease through February 2019. We also lease certain office equipment under operating leases.

In September 2009, we entered into a $25 million secured debt facility with a syndicate of two lenders. We borrowed $12.5 million under the debt facility in September 2009 and an additional $10 million under the facility in December 2010 and issued the lenders promissory notes. The notes are secured by substantially all our assets except for intellectual property. The notes carry a fixed interest rate of 13.65%. As of December 31, 2012, the outstanding principal balance on the notes was $4,752,000. The notes are scheduled to be repaid in full in January 2014. The debt facility contains negative covenants restricting our activities, including limitations on dispositions, mergers or acquisitions, incurring indebtedness or liens, paying dividends or making investments and certain other business transactions. There are no financial covenants associated with the debt facility. The debt facility contains certain events of default. The obligations under the debt facility and the other loan documents may at the lenders' option be accelerated upon the occurrence of certain events of default, and are automatically accelerated upon certain bankruptcy and insolvency related events of default.

The preceding table excludes contingent contractual payments that we may become obligated to make. Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products, including the following agreements with The Wellcome Trust Limited, or Wellcome Trust, and SMA Foundation.

We have entered into funding agreements with Wellcome Trust for the research and development of small molecule compounds in connection with our BMI1 and antibacterial programs. To the extent that we develop and commercialize program intellectual property on a for-profit basis, we may become obligated to pay to Wellcome Trust development and regulatory milestone payments of up to an aggregate of $68.9 million and single-digit royalties on sales of any research program product. Our obligation to pay

such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country.

We have also entered into a sponsored research agreement with the SMA Foundation in connection with our spinal muscular atrophy program. We may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that we successfully develop and subsequently commercialize or, with respect to collaboration products we outlicense, a specified percentage of certain payments we receive from our licensee. We are not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Our obligation to make such payments would end upon our payment to the SMA Foundation of a specified amount.

We have employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur.

Quantitative and qualitative disclosures about market risk

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase.

Recent accounting pronouncements

Effective January 1, 2012, an update to an accounting standard was issued that requires that all nonowner changes in stockholders' equity be presented either in a single continuous statement of comprehensive income (loss) or in two separate but consecutive statements. This update was applied retrospectively. The Company adopted this pronouncement and elected to present a separate statement of comprehensive income (loss). The updated standard does not change the items that must be reported in comprehensive income, how such items are measure, or when they must be reclassified to net income.

Business

Overview

We are a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes. While our discovery programs are directed at targets in multiple therapeutic areas, we are focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. Our lead product candidate is ataluren for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. We have retained worldwide commercialization rights to ataluren for all indications in all territories. We are initiating a Phase 3 clinical trial of ataluren for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD. We expect to dose the first patient in this trial in the first half of 2013. In October 2012, we submitted a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, for conditional approval of ataluren for the treatment of nmDMD. We are also planning a Phase 3 clinical trial of ataluren for the treatment of cystic fibrosis caused by nonsense mutations, or nmCF. We plan to initiate this trial in the second half of 2013, subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design. There are currently no marketed therapies approved to treat the underlying cause of nmDMD or nmCF. The EMA has designated ataluren as an orphan medicinal product and the U.S. Food and Drug Administration, or FDA, has granted orphan drug designation to ataluren for the treatment of both nmDMD and nmCF.

The letters "PTC" in our corporate name are an acronym for post-transcriptional control processes, which are the regulatory events that occur in cells after a messenger RNA, or mRNA, molecule is copied, or transcribed, from DNA. The mRNA molecules are key intermediates in protein production. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. The absence or overproduction of specific proteins can cause disease. The small-molecule compounds that we are developing are designed to alter post-transcriptional control processes to correct or compensate for a genetic defect. We apply proprietary technologies and our extensive knowledge of post-transcriptional control processes in our drug discovery and development activities. We believe that systematically targeting post-transcriptional control processes represents an unexploited approach to drug discovery and development.

We discovered ataluren by applying our technologies to identify molecules that promote or enhance the suppression of nonsense mutations. Nonsense mutations are implicated in a variety of genetic disorders. Nonsense mutations create a premature stop signal in the translation of the genetic code contained in mRNA and prevent the production of full-length, functional proteins. We believe that ataluren interacts with the ribosome, which is the component of the cell that decodes the mRNA molecule and manufactures proteins, to enable the ribosome to read through premature nonsense stop signals on mRNA and allow the cell to produce a full-length, functional protein. As a result, we believe that ataluren has the potential to be an important therapy for muscular dystrophy, cystic fibrosis and other genetic disorders for which a nonsense mutation is the cause of the disease. Genetic tests are available for many genetic disorders, including Duchenne muscular dystrophy and cystic fibrosis, to determine if the underlying cause is a nonsense mutation.

Muscular dystrophies involve progressive muscle wasting and weakness and are caused by a mutation in the DNA that results in either the absence or very low levels of the dystrophin protein. Duchenne muscular dystrophy is the most common and one of the most severe types of muscular dystrophy. Patients with

Duchenne muscular dystrophy typically lose walking ability by their early teens, require ventilation support in their late teens and, eventually, die due to heart and lung failure. The average age of death for Duchenne muscular dystrophy patients is in their mid-twenties.

Cystic fibrosis is caused by a mutation in the DNA that results in either the absence or very low levels of the cystic fibrosis transmembrane conductance regulator, or CFTR, protein. Cystic fibrosis results in the body producing abnormally thick and sticky mucus that clogs multiple organs, including the lungs, pancreas and liver. Cystic fibrosis leads to progressive loss of lung function, potentially life-threatening lung infections, permanent pancreatic damage and malnutrition. The average age of death for cystic fibrosis patients is in their mid-thirties. A nonsense mutation is a type of mutation in the DNA that can cause both Duchenne muscular dystrophy and cystic fibrosis.

We have completed a Phase 2b clinical trial of ataluren for the treatment of nmDMD and a Phase 3 clinical trial of ataluren for the treatment of nmCF. We did not achieve the primary efficacy endpoint in either trial with the pre-specified level of statistical significance. However, we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that ataluren was active and showed clinically meaningful improvements over placebo in these trials. In addition, we believe that our experience in these completed clinical trials has allowed us to enhance the designs of our confirmatory Phase 3 clinical trials and improve our likelihood of success in these trials. Accordingly, we are initiating a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD and are planning a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. Ataluren has been generally well tolerated in all of our clinical trials to date.

In October 2012, we submitted an MAA to the EMA for conditional approval of ataluren for the treatment of nmDMD. Although there is substantial risk that the EMA will not grant us this conditional approval, such approval would permit us to market ataluren in the European Union for treatment of nmDMD prior to completion of our confirmatory Phase 3 clinical trial for this indication. We plan to complete our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD before applying for marketing approval from the FDA. In designing our confirmatory Phase 3 clinical trial for the treatment of nmDMD, we have sought to reflect the views expressed by both the EMA and the FDA in our discussions with these regulatory authorities. We expect that these trial results, if favorable, could serve as the basis for full approval by the EMA and the FDA of ataluren for the treatment of nmDMD.

We also have received scientific advice from the EMA regarding the possibility of submitting an MAA for conditional approval of ataluren for the treatment of nmCF and our proposed trial protocol for a confirmatory Phase 3 clinical trial of ataluren for this indication. Subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design, we plan to pursue this additional conditional approval and, in the second half of 2013, to initiate the confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. There also is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of nmCF.

In addition to ataluren, we have a pipeline of product candidates that are in preclinical development. Our preclinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including neuromuscular disease, oncology and infectious disease. We have discovered all of our compounds currently under development using our proprietary technologies. We plan to develop these compounds both on our own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For example, we currently are collaborating with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation, for the development and commercialization of compounds for the

treatment of spinal muscular atrophy. Through December 31, 2012, we have received total collaboration funding of approximately $266 million and total grant funding and clinical trial support of approximately $87 million.

Our strategy

Our goal is to become a leading biopharmaceutical company focused on discovering, developing and commercializing small-molecule therapeutics that target post-transcriptional control processes and address disorders, particularly in the orphan and ultra-orphan areas, with high unmet medical needs. To achieve our goal, we are pursuing the following strategies:

- ***Complete development of and seek marketing approvals for ataluren in lead indications.*** We are devoting a significant portion of our resources and business efforts to completing the development of ataluren for the treatment of nmDMD and nmCF. We are initiating a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD and have applied for conditional approval to market ataluren for this indication in the European Union prior to completing this trial. We expect that these trial results, if favorable, could serve as the basis for full approval by the EMA and the FDA of ataluren for the treatment of nmDMD. Subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design, we plan to pursue conditional approval and, in the second half of 2013, to initiate a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF.

- ***Maximize commercial potential of ataluren.*** We have retained worldwide commercialization rights to ataluren for all indications in all territories. If ataluren receives marketing approval, we plan to commercialize it with our own focused, specialized sales force. We believe that the medical specialists treating Duchenne muscular dystrophy and cystic fibrosis are sufficiently concentrated that we will be able to effectively promote ataluren with targeted sales teams initially in the European Union and the United States and, eventually, in other key territories, such as Asia and Latin America.

- ***Explore additional indications for ataluren.*** We believe that ataluren has the potential to be an important therapy for other genetic disorders for which a nonsense mutation is the cause of the disease. We estimate that, on average, 11% of patients with any genetic disorder resulting from the absence of a single protein, referred to as monogenic disorders, have a nonsense mutation as the cause of the disease. We plan to select additional indications for further clinical development of ataluren consistent with the criteria that we applied in selecting nmDMD and nmCF, such as high unmet medical need and commercially available genotyping.

- ***Advance the development of our preclinical product candidates and continue to discover and develop small molecules that alter post-transcriptional control processes.*** Our preclinical and discovery programs are focused on new treatments for multiple therapeutic areas, including neuromuscular disease, oncology and infectious disease. We are particularly focused on the development and commercialization of treatments for orphan and ultra-orphan disorders. We are applying several proprietary technologies to identify, chemically optimize and develop small molecules designed to alter post-transcriptional control processes to achieve therapeutic effects. Because post-transcriptional control processes offer many targets for therapeutic intervention and because drugs that alter these processes have the potential to both increase and decrease protein production, we believe that our approach may be applicable to a broad range of diseases.

- ***Seek third party grants and support and selectively establish strategic alliances.*** We have obtained, and we intend to continue to seek, development funding and other assistance from government entities,

non-government and philanthropic organizations and patient advocacy groups for our product candidates. We previously have received grant funding and clinical trial support from the National Institutes of Health, the FDA, the Department of Defense, Defense Threat Reduction Agency, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, The Wellcome Trust Limited, or Wellcome Trust, Cystic Fibrosis Foundation Therapeutics and the SMA Foundation. In addition, for each of our product candidates, and in particular for product candidates that have high anticipated development costs, address markets requiring a large sales and marketing organization to serve effectively or are directed at indications for which a potential collaborator has a particular expertise, we plan to evaluate the merits of entering into collaboration arrangements with leading pharmaceutical or biotechnology companies.

Our product development programs

The following table summarizes key information about our most advanced product development programs. All of the compounds in these programs are new chemical entities that we identified using our proprietary technologies.

Program	Development status	Development and commercial rights
Ataluren for nmDMD	• Phase 2b clinical trial completed • Confirmatory Phase 3 clinical trial being initiated	PTC
Ataluren for nmCF	• Phase 3 clinical trial completed • Confirmatory Phase 3 clinical trial planned for second half of 2013	PTC
Spinal muscular atrophy	• Preclinical • Optimization of development compounds ongoing	Roche
Oncology—BMI1	• Preclinical • Lead development compound selected	PTC
Antibacterial	• Preclinical • Optimization of development compounds ongoing	PTC

Background on genetic disorders and nonsense mutations

A significant number of rare genetic disorders are monogenic disorders that occur as a consequence of the absence of a single protein. The restoration of the production of that single protein has the potential to treat the genetic disorder. We estimate that, on average, 11% of patients with any monogenic disorder have a nonsense mutation as the cause of the disease.

Through the post-transcriptional process of translation, a specialized cellular apparatus, called the ribosome, manufactures functional proteins by translating the genetic code contained in the mRNA. This decoding process reads the building blocks of the mRNA, known as nucleotides, in groups of three. Each group of three nucleotides is called a codon. Three of the 64 possible codons contained in mRNA serve as normal stop signals and indicate the end of the protein-coding region of the mRNA. When functioning properly, the stop codons cause the ribosome to halt translation of the mRNA once the mRNA's genetic code has been completely translated into a full-length, functional protein.

There are four basic types of mutations in DNA that can cause a genetic disorder. These are known as insertion, deletion, missense and nonsense mutations. A nonsense mutation is a single nucleotide alteration in the DNA that, when copied to mRNA, is interpreted by the ribosome as a premature stop signal and terminates translation within the protein-coding region of the mRNA. When a ribosome encounters a premature stop codon, the translation process is terminated before a full-length, functional protein is formed. The resulting truncated protein is usually unstable and unable to serve its necessary function. The absence of a full-length, functional protein may cause disease.

Cells have a mechanism that discriminates a normal stop codon from a premature stop codon, although both types of stop codon result in termination of the translation of the genetic code. A group of proteins, known as the termination surveillance complex, can discriminate the proteins downstream of a normal stop codon to regulate normal translational termination. Because these proteins do not appear to be downstream of a premature stop codon, a normal stop codon can be distinguished from a premature stop codon.

Ataluren

Overview

Ataluren is a novel, orally administered small-molecule compound that targets nonsense mutations. We are developing ataluren for the treatment of genetic disorders in which a nonsense mutation is the cause of the disease. We believe that a drug with a mechanism of action that allows the ribosome to read through premature stop codons without affecting the normal termination of protein synthesis may be able to overcome the effects of nonsense mutations.

Ataluren allows the cellular machinery to read through premature stop codons in mRNA and enable the translation process to produce full-length, functional proteins. As described above, certain factors that are located downstream of a normal stop codon are not present at a premature stop codon. We believe that these factors allow ataluren to be active only at premature stop codons without allowing ataluren to read through normal stop codons. Ataluren is from a distinct structural class that does not have antibiotic properties and we believe acts at a different location on the ribosome than gentamicin. Ataluren has been generally well tolerated in all of our clinical trials to date, which involved approximately 600 individuals dosed with ataluren.

The EMA has designated ataluren as an orphan medicinal product for the treatment of nmDMD and nmCF. The FDA has granted orphan drug designation and fast track designation to ataluren for the treatment of nmDMD and nmCF. There are currently no marketed therapies approved to treat the underlying cause of nmDMD or nmCF.

The following table sets forth information regarding our completed, ongoing and planned Phase 2 and Phase 3 clinical trials of ataluren for the treatment of nmDMD and nmCF.

Phase 2 and Phase 3 clinical trials of ataluren for nmDMD and nmCF

Study	Phase, study design, location	Total patients enrolled	Status	Dates
nmDMD				
nmDMD-004	Phase 2a, open label, United States	38	Completed	December 2005 to May 2007
nmDMD-004e	Phase 2a extension, open label, United States	36 (patients previously in nmDMD-004)	Ended	August 2008 to May 2010
nmDMD-008	Phase 2a, open label, United States	6	Ended	January 2010 to March 2010
nmDMD-007	Phase 2b, double-blind, placebo controlled, Australia, Canada, European Union, Israel, United States	174	Completed	February 2008 to December 2009
nmDMD-007e	Phase 2b extension, open label, Australia, Canada, European Union, Israel, United States	173 (patients previously in nmDMD-007)	Ended	January 2009 to May 2010
nmDMD-016	Phase 3 continuation, open label, United States	Up to 122 (patients previously in nmDMD-004 or nmDMD-007)	Ongoing	Initiated in November 2010
nmDMD-019	Phase 3 continuation, open label, Australia, Canada, European Union, Israel	Up to 96 (patients previously in nmDMD-004)	Ongoing	Initiated in May 2012
nmDMD-020	Confirmatory Phase 3, double-blind, placebo controlled, planned as Australia, Canada, European Union, Israel, South America, United States	Approximately 220	Enrolling trial sites	First patient expected to be dosed in first half of 2013

Study	Phase, study design, location	Total patients enrolled	Status	Dates
nmCF				
nmCF-003	Phase 2, open label, United States	24	Completed	November 2005 to December 2006
nmCF-005	Phase 2, open label, Israel	23	Completed	November 2005 to May 2006
nmCF-005e	Phase 2a extension, open label, Israel	19 (patients previously in nmCF-005)	Completed	January 2007 to June 2007
nmCF-006	Phase 2a, open label, Belgium, France	30	Completed	March 2007 to February 2008
nmCF-009	Phase 3, double-blind, placebo controlled, Canada, European Union, Israel, United States	238	Completed	September 2009 to November 2011
nmCF-009e	Phase 3 extension, open label, Canada, European Union, Israel, United States	191 (patients previously in nmCF-009)	Ongoing	Initiated in August 2010
nmCF-021	Confirmatory Phase 3, double-blind, placebo controlled, global trial sites planned	Approximately 210	Planned	Plan to initiate in second half of 2013

We have completed a Phase 2b clinical trial of ataluren for the treatment of nmDMD and a Phase 3 clinical trial of ataluren for the treatment of nmCF. We did not achieve the primary efficacy endpoint in either trial with the pre-specified level of statistical significance. However, we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that ataluren was active and showed clinically meaningful improvements over placebo in these trials. Accordingly, we are initiating a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD and are planning a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. We believe that our experience in our completed clinical trials has allowed us to enhance the designs of our confirmatory Phase 3 clinical trials and improve our likelihood of success. We plan to initiate our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF in the second half of 2013, subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design.

Ataluren is administered orally as granules mixed with permitted liquids or semi-solid foods, such as milk, water, applesauce or yogurt. We designed this formulation because children comprise a significant portion of the patient population for ataluren and often have difficulty swallowing pills or capsules. Ataluren is manufactured in reliable and reproducible synthetic processes from readily available starting materials. Ataluren has been generally well tolerated to date in our Phase 2 and Phase 3 clinical trials.

Duchenne muscular dystrophy

Muscular dystrophies are genetic disorders involving progressive muscle wasting and weakness. Duchenne muscular dystrophy is the most common and one of the most severe types of muscular dystrophy.

Duchenne muscular dystrophy occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Dystrophin is a muscle membrane associated protein and is critical to the structural and membrane stability of muscle fibers in skeletal, diaphragm and heart muscle. The absence of normally functioning dystrophin results in muscle fragility, such that muscle injury occurs when muscles contract or stretch during normal use. As muscle damage progresses, connective tissue and fat replace muscle fibers, resulting in inexorable muscle weakness.

Because the dystrophin gene is located on the X chromosome, Duchenne muscular dystrophy occurs almost exclusively in young boys. According to Parent Project Muscular Dystrophy, Duchenne muscular dystrophy occurs in approximately 1 in 3,500 live male births. Based on this prevalence data, we estimate that Duchenne muscular dystrophy affects a total of approximately 15,000 boys and adolescents in the United States. Based on data from Orphanet, a public reference portal for information on rare disorders and orphan drugs, we estimate that Duchenne muscular dystrophy affects a total of approximately 19,000 boys and adolescents in the European Union. Genetic tests are available to determine if a patient's Duchenne muscular dystrophy is caused by a nonsense mutation. Based on information from Prior, et al. (1995) in the American Journal of Human Genetics, we estimate that a nonsense mutation is the cause of Duchenne muscular dystrophy in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union.

Children with Duchenne muscular dystrophy typically begin to show symptoms as early as age three, when they develop a waddling gait, may seem clumsy, frequently fall and have difficulty rising from the floor. Progressive weakness then develops in the voluntary muscles in the arms, legs and trunk. This muscle weakness results in fixations, or contractures, of joints, such as knees, hips, elbows and feet. By the age of eight, most patients have difficulty ascending stairs. By their early teens, patients typically lose walking ability and are confined to wheelchairs. Patients' hearts and respiratory muscles are also affected, typically requiring use of ventilators in their late teens. Further progressive loss of strength and the weakening of heart and lung muscles eventually results in death due to heart and lung failure. The average age of death for Duchenne muscular dystrophy patients is in their mid-twenties.

There is currently no marketed therapy approved for the treatment of the underlying cause of Duchenne muscular dystrophy. Currently available treatments for Duchenne muscular dystrophy are only palliative. These treatments seek to address the symptoms through supportive care measures, such as bracing to give patients some opportunity to remain standing, joint stretching exercises to avoid contractures and tendon release surgery. Corticosteroids are prescribed to mitigate the symptoms of the disease but can cause significant complications because of chronic toxicities. We believe that no other therapy in clinical development for Duchenne muscular dystrophy is designed to treat the underlying cause of nmDMD.

Planned Phase 3 clinical trial of ataluren for nmDMD

We are currently enrolling trial sites for a multicenter, randomized, double-blind, placebo controlled Phase 3 clinical trial to evaluate the efficacy and safety of ataluren in patients with nmDMD as confirmed by gene sequencing. We expect to dose the first patient in this trial in the first half of 2013. We plan to conduct this trial in approximately 220 patients at investigational sites worldwide.

The primary objective of this trial will be to evaluate the effect of ataluren on ambulation. The primary efficacy endpoint specified in our trial protocol is mean change from baseline over 48 weeks in distance walked during a 6-minute walk test, which we refer to as 6-minute walk distance. The 6-minute walk test is well established as an endpoint for a number of different rare and orphan diseases involving muscle wasting and weakness. Following completion of our Phase 2b clinical trial described below, the 6-minute

walk test has become the most common primary endpoint currently used in Duchenne muscular dystrophy clinical trials.

Supportive analyses of ambulation in our trial protocol consist of:

- proportion of patients with at least 10% worsening in 6-minute walk distance at week 48 of the trial compared to baseline;
- time from baseline to persistent 10% worsening in 6-minute walk distance; and
- change from baseline in percent of predicted 6-minute walk distance compared to healthy boys matched for age and height, which we refer to as %-predicted 6-minute walk distance.

Secondary endpoints in the trial consist of change in timed tests of muscle function based on time to climb four stairs, descend four stairs and run/walk 10 meters. Timed function tests are well established in the clinical evaluation of Duchenne muscular dystrophy. Restoration of dystrophin stabilizes muscle membranes, so that the integrity of muscle fibers is maintained, but does not directly increase muscle strength. As a result, we believe that timed function tests provide a more sensitive measure of treatment effect than measures of muscle strength. In addition, because many Duchenne muscular dystrophy patients have very low baseline muscle strength, it is difficult to demonstrate a difference in the rate of decline of muscle strength in these patients.

The trial protocol also includes two secondary endpoints that have not been used previously as outcome measures in published therapeutic clinical trials. The first new endpoint is a functional scale specifically designed for ambulant Duchenne muscular dystrophy patients, referred to as the North Star Ambulatory Assessment, or NSAA. The NSAA is a composite of muscle function tests, such as the ability to rise from the floor, ability to get from lying to sitting, ability to get from sitting to standing and ability to hop, jump and run. The other new endpoint captures patient-reported changes in activities of daily living based on a disease symptom survey that we developed.

The trial protocol specifies the following key inclusion criteria for patients enrolling in the trial:

- the patient must be seven through 16 years of age;
- at baseline, the patient must walk no more than 80% of predicted 6-minute walk distance compared to healthy boys matched for age and height, but have the ability to walk at least 150 meters during the 6-minute walk test; and
- the patient must have used systemic corticosteroids for a minimum of six months prior to start of treatment.

The trial protocol provides for the exclusion of patients from the trial if they have a prior or ongoing clinically significant illness, recently used systemic aminoglycoside antibiotics, recently initiated or changed corticosteroid therapy or previously received ataluren treatment. We will perform study assessments at clinic visits every eight weeks. Patients will undergo 48 weeks of blinded treatment prior to the final analysis.

We plan to stratify patients in this trial based on age, baseline 6-minute walk distance and duration of prior use of corticosteroids. The trial protocol provides that patients will be randomized in a 1:1 ratio to receive either placebo or ataluren at a dosing regimen consisting of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg. This was the same 10, 10, 20 dose of ataluren that showed beneficial results in our completed Phase 2b clinical trial described below.

Based on our plan to dose the first patients in this trial in the first half of 2013 and our estimates regarding patient enrollment, we expect to complete this trial and have initial, top-line data available in mid-2015. At the completion of blinded treatment, an open label continuation trial will be available to patients who successfully complete the trial in countries where ataluren is not commercially available at that time. Patients in the continuation trial will receive ataluren in the same dosing regimen as in the confirmatory Phase 3 clinical trial.

Rationale for design of Phase 3 clinical trial of ataluren for nmDMD

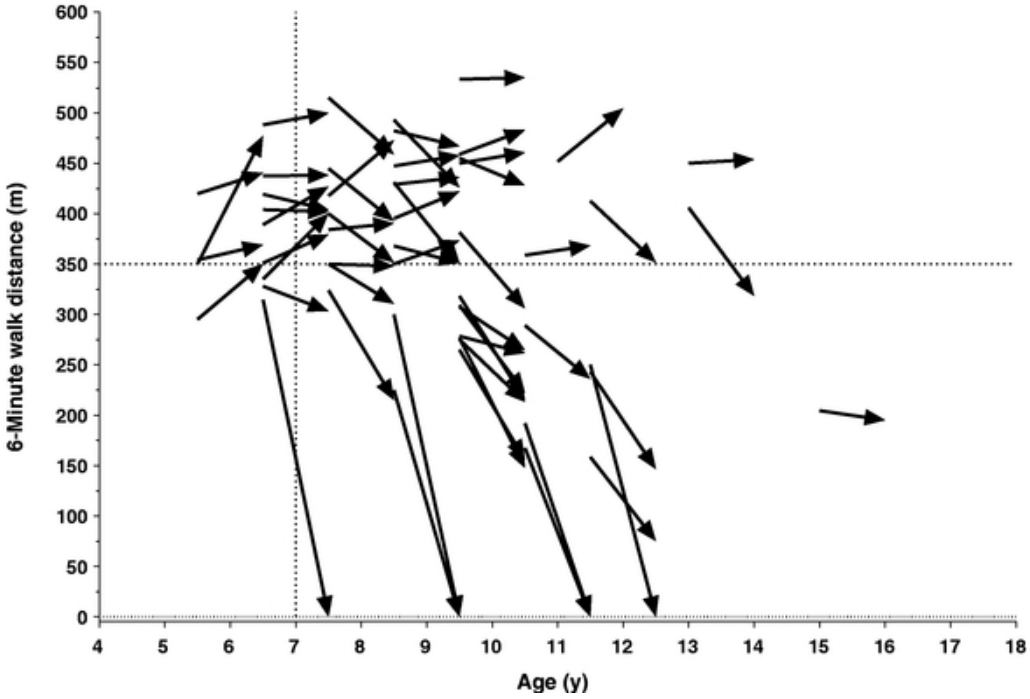
The study population and outcome measures that we are using in our confirmatory Phase 3 clinical trial are based on, and reflect our analysis of the results of, our completed Phase 2b clinical trial for the treatment of nmDMD, including data regarding disease progression, referred to as natural history data, based on patient age and baseline walking ability. Specifically, in our Phase 2b clinical trial:

- Patients who were younger than seven years of age tended to have stable or increasing 6-minute walk distance over 48 weeks. We believe that this reflects the fact that growth and development predominate over disease progression at these ages. Patients seven years of age and older typically had declining 6-minute walk distance over 48 weeks, indicating that they were in the decline phase of the disease. Accordingly, to focus on patients likely to be in the decline phase of the disease, our Phase 3 clinical trial design requires that patients be at least seven years of age.

- The 6-minute walk distance for patients at least seven years of age decreased at different rates over 48 weeks depending on their baseline 6-minute walk distance. Patients whose baseline 6-minute walk distance was greater than 350 meters tended to have stable 6-minute walk distance over 48 weeks. Patients with baseline 6-minute walk distance of less than 350 meters generally declined over 48 weeks, some to the point of becoming non-ambulatory. Accordingly, to focus on patients likely to be in the decline phase of the disease, our Phase 3 clinical trial design requires that patients must walk no more than 80% of predicted 6-minute walk distance compared to healthy boys matched for age and height.

Natural history data from patients in the placebo group in our Phase 2b clinical trial, based on age and baseline 6-minute walk distance, are illustrated in the figure below, in which each placebo-treated patient from the trial is represented by an arrow. The base of the arrow indicates the patient's 6-minute walk distance at baseline, and the tip of the arrow indicates this measurement at week 48 of the trial.

Natural history results from placebo-treated patients in Phase 2b clinical trial



In addition, as discussed in more detail below, we performed a post-hoc, retrospective subgroup analysis of patients from our completed Phase 2b clinical trial who would meet the enrollment criteria for our confirmatory Phase 3 clinical trial. This analysis showed a much larger treatment effect in mean change in 6-minute walk distance over 48 weeks between ataluren and placebo in this subgroup than in the overall population included in the Phase 2b clinical trial.

In light of the natural history data from our Phase 2b clinical trial and this retrospective subgroup analysis, our confirmatory Phase 3 clinical trial will focus on patients in the decline phase of the disease based on age and baseline 6-minute walk distance. The intent of focusing on patients in the decline phase of the disease is to enhance the demonstration of ataluren's effect to slow decline in walking ability. In addition, we believe that by only enrolling patients who are being treated with systemic corticosteroids, the variability of 6-minute walk distance results will be reduced. Notwithstanding that we expect a larger treatment effect and less variability of results in our Phase 3 clinical trial than in our Phase 2b clinical trial, the sample size of patients in our Phase 3 clinical trial is designed to be large enough to achieve statistical significance even if we achieve the same treatment effect and similar variability as in our Phase 2b clinical trial.

Regulatory status and strategy for nmDMD

EMA. On October 30, 2012, we submitted an MAA to the EMA for conditional approval of ataluren for the treatment of nmDMD. The EMA validated the MAA submission on November 21, 2012, which initiated the formal EMA review process of the MAA. EMA conditional approval would permit us to market ataluren in the European Union for nmDMD prior to the completion of our planned Phase 3 clinical trial. Conditional approval is valid for one year, with annual renewals required thereafter. Upon granting conditional approval, the EMA specifies the obligations and the timeframe to fulfill them for subsequent full approval.

The EMA will consider conditional marketing approval of a product candidate that is being developed to treat a seriously debilitating or life-threatening disease or that is designated as an orphan medicinal product notwithstanding that one or more additional pivotal clinical trials may be required for full approval. Such a product candidate must satisfy each of the four requirements listed below.

- *The risk-benefit balance of the product candidate must be positive*. We believe that the efficacy data from our completed Phase 2b clinical trial of ataluren, although not statistically significant based on the pre-specified methodology for analyzing the data, together with the strong safety data from clinical trials of ataluren conducted to date, indicate that the benefits of ataluren outweigh its risks in this patient population. We believe that in this Phase 2b clinical trial ataluren demonstrated, particularly based on post-hoc refined analyses that were not pre-specified in the clinical trial protocol, clinically meaningful treatment effects on 6-minute walk distance and timed tests of stair-climbing, stair-descending and running/walking 10 meters. These treatment effects are supported by other outcome measures of physical functioning, such as activity and wheelchair use in the community setting, frequency of accidental falling and patient-reported physical functioning.

- *The applicant must be likely to provide comprehensive data*. We plan to dose the first patient in our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD in the first half of 2013. We expect that patient enrollment in this trial will be complete or nearly complete prior to the completion of regulatory review and frequently lengthy reimbursement processes relating to our conditional approval application, with a substantial proportion of patients having finished the treatment period prior to completion of these processes. Although it is possible that some late-enrolled patients in the European Union may drop out of the trial if ataluren becomes commercially available following conditional approval, we believe that this risk is small, and that the effect on the trial would be minimal because we expect a large number of patients to be enrolled in countries outside the European Union. We also believe that clinical investigators, who may also be the primary physicians for patients in the trial, and patient advocacy groups will encourage patients to remain in the trial.

- *The product candidate must fulfill an unmet medical need*. There are currently no marketed therapies approved to treat the underlying cause of nmDMD. Currently available treatments are only palliative.

- *The benefits to public health of the immediate availability of the product candidate must outweigh the risks inherent in the fact that additional data are still required.* We believe that the benefits of immediate availability of ataluren outweigh the risks of conditional approval for patients with nmDMD. If marketing approval were delayed until comprehensive Phase 3 clinical data are available and patients with nmDMD are not treated, these patients will suffer irreversible loss of function.

The EMA informed us in scientific advice that it provided in May 2012 that although ataluren falls within the scope of the regulation for conditional approval, it appeared unlikely that a positive risk-benefit assessment for ataluren could be concluded primarily based on the results of our completed Phase 2b clinical trial. In this Phase 2b clinical trial, we only approached statistical significance on the primary efficacy endpoint in post-hoc refined analyses that were not pre-specified in the trial protocol, and efficacy results were partly inconsistent in secondary efficacy endpoints. In addition, the EMA questioned whether it is practical to believe that a Phase 3 clinical trial for this indication could be completed if the EMA grants conditional approval, which would reduce the likelihood that we could provide comprehensive data following approval. However, the EMA also stated that the rareness of the disease is an important consideration. In addition, the EMA informed us that it is important that the confirmatory trial be well-advanced by the time of the conditional approval decision, which we expect to be able to accomplish based on our development plan described above.

After receiving the scientific advice from the EMA, we held meetings with the rapporteur and the co-rapporteur for regulatory review of ataluren for the treatment of nmDMD. The role of the rapporteur is to perform the scientific evaluation of an application and prepare an assessment report for the EMA. The co-rapporteur prepares an independent assessment report or provides a critique of the rapporteur's report. In our meetings, the rapporteur and the co-rapporteur indicated to us that they support 6-minute walk distance as the primary endpoint and that an endpoint to measure muscle strength would not be required in the proposed confirmatory Phase 3 clinical trial, as had been previously suggested in the scientific advice from the EMA.

Based on recommendations from the EMA and further advice from the rapporteur and the co-rapporteur, we addressed the following matters in our MAA:

- the mechanism of action of ataluren;
- the clinical relevance of the 30-meter difference in 6-minute walk distance in Duchenne muscular dystrophy;
- the lack of dystrophin quantification in our Phase 2b clinical trial;
- the bell-shaped dose-response in our Phase 2b clinical trial; and
- the reasons for performing the post-hoc analyses of the data.

Although there is substantial risk that the EMA will not grant us this conditional approval, we are pursuing this approach because we believe the drug is active and should be made commercially available as soon as possible to patients who have no other treatment options. We plan to complete our planned Phase 3 clinical trial of ataluren for the treatment of nmDMD even if the EMA does not grant conditional approval. We expect that these trial results, if favorable, could serve as the basis for full marketing approval in the European Union, the United States and other territories.

FDA. Following a meeting with the FDA in November 2009 in which we discussed our submission of a new drug application, or NDA, we submitted to the FDA the final component of an NDA in March 2011 for approval of ataluren for the treatment of nmDMD. The FDA refused to file this NDA on the grounds that the single placebo controlled Phase 2b clinical trial contained in the NDA did not achieve statistical significance in the pre-specified analysis. In December 2011, we filed with the FDA a formal dispute resolution request concerning the NDA. We requested review of the issues related to the FDA's refusal to file the NDA and a prospective resubmission of the NDA with updated information and analyses. In January 2012, the FDA reaffirmed the appropriateness of its earlier decision to refuse to file the NDA. In February 2012, we discussed the design of a proposed Phase 3 clinical trial with the FDA. At that time, the FDA indicated that the adequacy of data for filing and approval of an NDA remain review issues, but that the study design for the proposed clinical trial, in general, was appropriate for providing evidence of efficacy of ataluren. Consequently, we plan to include the safety and efficacy data from our confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD as part of our application for marketing approval from the FDA if we successfully complete this trial.

Phase 2b clinical trial of ataluren for nmDMD

In March 2010, we announced the results of a randomized, double-blind, placebo controlled, dose ranging Phase 2b clinical trial evaluating the long term efficacy and safety of ataluren in patients with nmDMD as confirmed by gene sequencing. We conducted this clinical trial in 174 patients at 37 investigational sites in 11 countries. Before this clinical trial, there was no established precedent for an appropriate trial design to

evaluate the efficacy of ataluren and little clinical experience in the methodologies used to analyze the resulting data. In addition, at the time we designed and initiated this trial, our knowledge of the natural history of Duchenne muscular dystrophy patients was limited. In particular, there were no data available regarding change in 6-minute walk distance over time for patients with Duchenne muscular dystrophy. As a result of this trial, we improved our understanding of the patient population likely to demonstrate the greatest measurable benefit from treatment, the dose of ataluren most likely to demonstrate efficacy and the appropriate statistical plan for analyzing the trial data.

The primary objective of this trial was to evaluate the effect of ataluren on ambulation. The primary efficacy endpoint was the mean change in 6-minute walk distance at week 48 of the trial compared to baseline. Supportive analyses of ambulation consisted of the proportion of patients with at least 10% worsening in 6-minute walk distance at week 48 of the trial compared to baseline and time to persistent 6-minute walk distance 10% worsening from baseline.

We included many secondary and exploratory endpoints in this trial to gain greater understanding of clinical trial design in Duchenne muscular dystrophy and not with the sole objective of showing a treatment effect. Secondary endpoints in the trial included monitoring changes in:

- tests of muscle function based on time to climb four stairs, descend four stairs, run/walk 10 meters and stand from supine;
- muscle strength;
- patient/caregiver reported frequency of accidental falls;
- patient/caregiver reported health related quality of life;
- patient/caregiver reported treatment satisfaction;
- at-home activity as measured by pedometry;
- verbal memory and attention;
- heart rate function;
- creatine kinase, or CK, values as a measure of whole-body muscle fragility; and
- biceps muscle dystrophin expression.

We assessed safety through collection of adverse event information, measurement of laboratory parameters and performance of electrocardiograms, or ECGs. We also evaluated study drug compliance and ataluren plasma concentrations.

Patients enrolled in this trial were at least five years of age, had the ability at baseline to walk at least 75 meters unassisted during a 6-minute walk test, had onset of disease signs/symptoms prior to age nine, had elevated CK levels and had ongoing difficulty with walking. Patients were excluded from the trial if they had a prior or ongoing clinically significant illness, had a positive hepatitis B or hepatitis C test or had recently used systemic aminoglycosides. Patients receiving corticosteroid therapy were required to be on a stable dosing regimen for at least six months prior to entering the trial. The trial protocol specified a clinic visit every six weeks to assess efficacy and safety and an interim laboratory visit every three weeks for the first 24 weeks of the trial. The treatment duration was 48 weeks.

We stratified patients in this trial based on age, baseline 6-minute walk distance and use of corticosteroids. Patients were randomized in a 1:1:1 ratio to receive one of the following:

- placebo;
- ataluren at a dosing regimen consisting of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg, which we refer to as the 10, 10, 20 dose of ataluren; or
- ataluren at a dosing regimen consisting of 20 mg/kg in the morning, 20 mg/kg at midday and 40 mg/kg in the evening, for a total daily dose of 80 mg/kg, which we refer to as the 20, 20, 40 dose of ataluren.

Pre-specified analysis in ITT population. As specified in the trial protocol, we performed the primary analysis of the mean change in 6-minute walk distance from baseline to 48 weeks in the intent-to-treat, or ITT, population. The ITT population included all 174 randomized patients with a valid 6-minute walk test available at baseline and at least one post-baseline visit.

In this trial, the patients taking the 10, 10, 20 dose of ataluren had notably less decline in their walking ability than the patients taking placebo. The mean change from baseline to 48 weeks in 6-minute walk distance was -42.6 meters, with a standard deviation from the mean of 90.0 meters, in the placebo group, and -12.9 meters, with a standard deviation from the mean of 72.0 meters, in the ataluren 10, 10, 20 dose group. The difference of 29.7 meters between the 10, 10, 20 dose of ataluren and placebo in mean change in 6-minute walk distance over 48 weeks was consistent with the clinically meaningful treatment effect of 30 meters specified in the trial protocol. However, the resulting nominal p-value of 0.149 was not statistically significant at the pre-specified level of less than 0.05. We had targeted a treatment effect of 30 meters because marketed drugs for other genetic disorders that affect muscle activity were approved on the basis of a difference of approximately 30 meters in 6-minute walk distance.

Typically, a trial result is statistically significant if the chance of it occurring when the treatment is like placebo is less than one in 20, resulting in a p-value of less than 0.05. A p-value is called nominal if it is the result of one particular comparison when more than one comparison is possible, such as when two active treatments are compared to placebo or when two or more subgroups are analyzed.

We believe that the principal reasons for the lack of statistical significance for the primary efficacy endpoint in this trial, notwithstanding having achieved the targeted treatment effect, were the higher than expected mean variability in the results of individual patients over 48 weeks, as measured by the standard deviation from the mean, and the heterogeneous population of nmDMD patients based on age and baseline 6-minute walk distance. We believe that the high standard deviation in the 6-minute walk distance data resulted from the substantial variability of disease progression in Duchenne muscular dystrophy in patients in the wide age range that we enrolled in this trial. In particular, we believe that younger patients and patients with higher baseline 6-minute walk distances are less likely to exhibit measurable declines in 6-minute walk distance over 48 weeks. Based on information available at the time we designed this trial, we selected the sample size of the trial based on an assumed standard deviation of 50 meters and enrolled patients between five and 20 years of age. However, the higher actual standard deviation in the trial of between 72 and 90 meters made it difficult to achieve statistical significance without a larger patient population.

In this trial, there was no difference between placebo and the 20, 20, 40 dose of ataluren in the mean change in 6-minute walk distance over 48 weeks. Although unanticipated, this finding is consistent with a

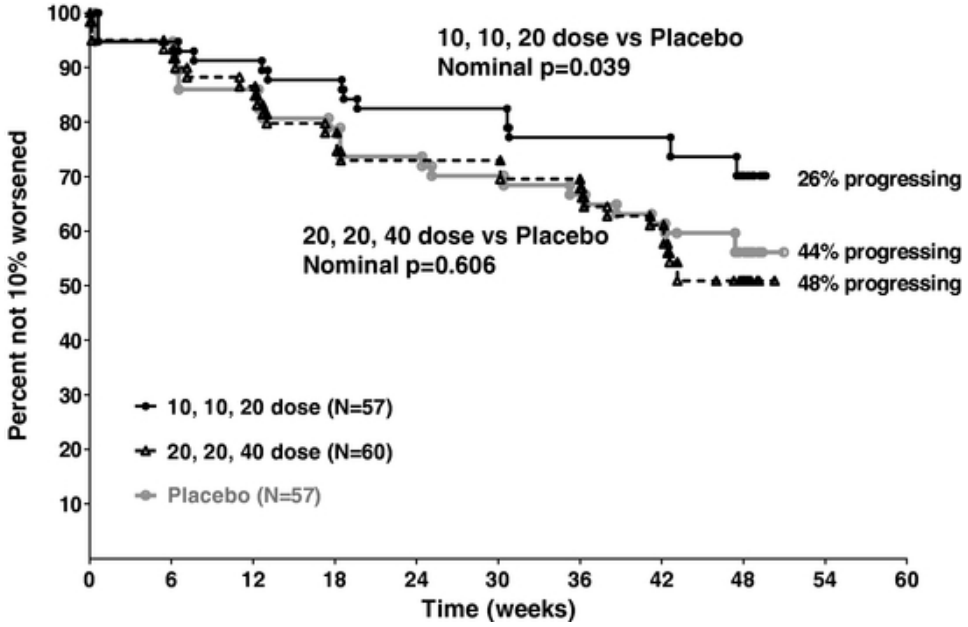
bell-shaped dose-response curve that we observed in four subsequent non-clinical studies of ataluren in Duchenne muscular dystrophy and other genetic disorders.

Pre-specified supportive analyses of ambulation. The protocol for our Phase 2b clinical trial included the following two supportive analyses of ambulation:

- an evaluation of the proportion of patients with at least 10% persistent worsening in 6-minute walk distance at week 48 compared to baseline; and
- time to persistent 6-minute walk distance 10% worsening from baseline.

The 10% persistent worsening threshold was defined in advance and reflects the clinical meaningfulness of a 10% change in walking ability in Duchenne muscular dystrophy. Specifically, a change of 10% in walking ability in one year is generally predictive of substantial decline in a patient's clinical status over the following years. The proportion of patients with at least 10% persistent worsening in 6-minute walk distance over the course of the trial is shown in the graph below.

Proportion of patients with persistent 10% worsening in 6-minute walk distance from baseline to week 48 (ITT population)



We believe that this analysis of the 6-minute walk distance indicates a meaningful delay in decline in ambulation for the 10, 10, 20 dose of ataluren compared to placebo and supports the primary analysis of mean change from baseline in 6-minute walk distance in the ITT population.

The analysis of time to 10% persistent worsening indicated that the pre-specified median time to 10% worsening was not reached in any of the three treatment arms of the trial.

Post-hoc analyses of Phase 2b clinical trial data. Based on our further evaluation of the data from our Phase 2b clinical trial after unblinding the results, we identified three issues affecting the pre-specified

statistical analyses. We addressed these issues in a post-hoc, retrospective refinement to the pre-specified statistical analysis plan, resulting in what we refer to as a corrected ITT analysis.

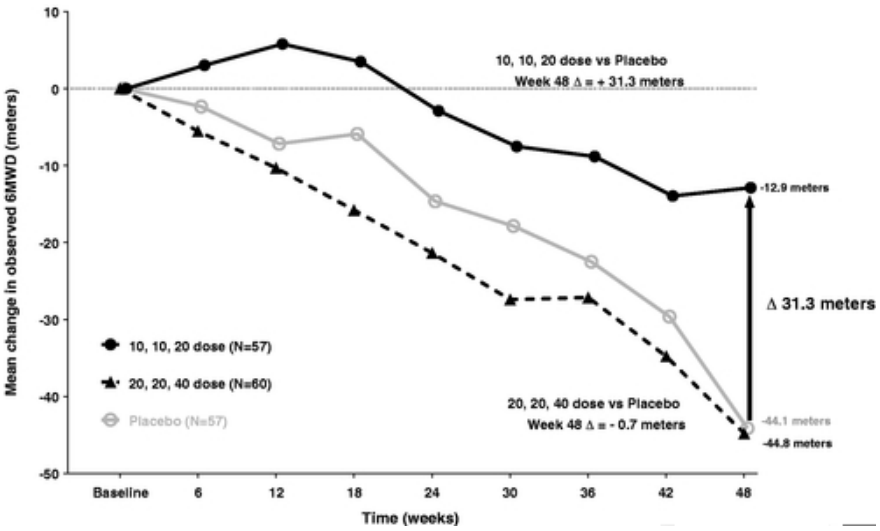
- Our pre-specified statistical model used to calculate the p-value and significance of the trial results omitted a specific statistical term designed to address the potential relationship between the 6-minute walk distance results at baseline and at each subsequent patient visit. As has now become standard practice in analyses of repeated-measures data, we adjusted our statistical model to add this statistical term in preparing the corrected ITT analysis.

- Because the 6-minute walk distance data were non-normally distributed, our pre-specified analysis used rank-transformed data in which the 6-minute walk distance values for each patient were ordered from smallest to largest and ranked from one to 174. However, ranking the data in this way did not fully reflect the large variability as measured in meters that we observed in the original 6-minute walk distance data. In the corrected ITT analysis, we used a re-randomization test, rather than rank transformation of the data, to address non-normality of the trial data. This re-randomization test allowed analysis of the 6-minute walk distance results in meters, rather than ranking the results relative to one another, to more accurately reflect the large variability in walking distances.

- Two patients had lower limb injuries after screening but prior to their baseline assessment. These injuries substantially affected their walking ability and led to aberrantly low baseline 6-minute walk distance values that did not accurately reflect their pre-treatment ambulatory ability. These baseline 6-minute walk tests were incorrectly classified as valid by the investigative site, and the resulting data should not have been included in the ITT analysis. In the corrected ITT analysis, we replaced the baseline values for these two patients with their valid screening values.

The results of our post-hoc analysis of the primary efficacy endpoint of this trial are shown in the graph and the table below.

Mean change in 6-minute walk distance (6MWD) by visit (corrected ITT analysis)



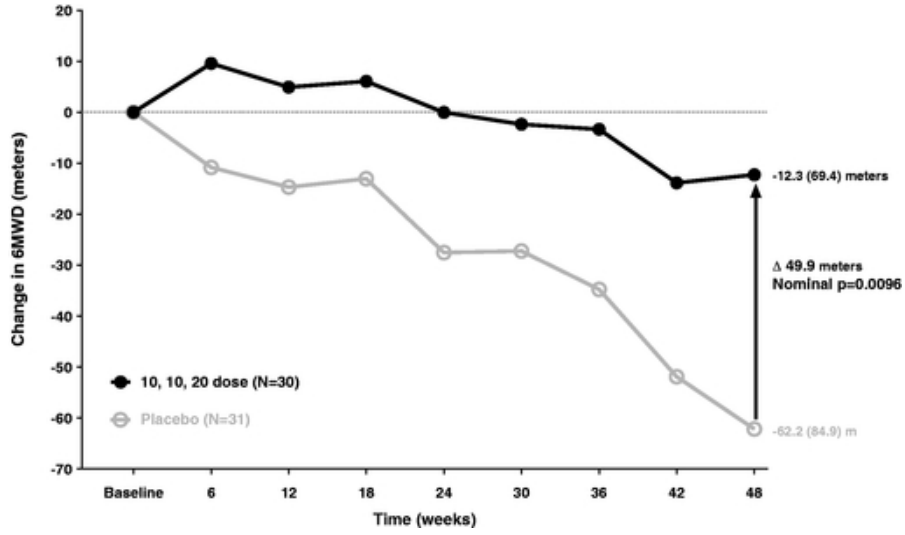
Change in 6-minute walk distance from baseline to week 48 (corrected ITT analysis)

	Treatment arm		
Summary of change from baseline to week 48	Placebo N=57	Ataluren 10, 10, 20 dose N=57	Ataluren 20, 20, 40 dose N=60
Mean (standard deviation), meters	-44.1 (88.0)	-12.9 (72.0)	-44.8 (84.8)
Mean difference from placebo, meters		31.3	-0.7
Nominal p-value (vs. placebo)		0.0281	0.912
Adjusted p-value (vs. placebo)		0.0561	0.991

In the corrected ITT analysis, the difference between the 10, 10, 20 dose of ataluren and placebo in mean change in 6-minute walk distance over 48 weeks was 31.3 meters. We observed clear separation between the 10, 10, 20 dose of ataluren and placebo, with the difference between the arms increasingly favoring the 10, 10, 20 dose of ataluren over time. The resulting nominal p-value for the comparison of mean change in 6-minute walk distance from baseline to week 48 for the 10, 10, 20 dose of ataluren versus placebo was 0.0281. However, because two dose levels were compared to placebo, we were required to apply a multiplicity adjustment, which yielded a final adjusted p-value of 0.0561 for the 10, 10, 20 dose of ataluren versus placebo. Regulatory authorities typically give greatest weight to results from pre-specified analyses and adjusted p-values and less weight to results from post-hoc, retrospective analyses and nominal p-values.

Subgroup analysis based on enrollment criteria for confirmatory Phase 3 clinical trial. Using the corrected ITT analysis, we also performed a post-hoc, retrospective subgroup analysis of patients in the Phase 2b clinical trial who would meet the enrollment criteria for our confirmatory Phase 3 clinical trial. We expect that these patients would be in the decline phase of the disease, based on age and baseline 6-minute walk distance. Patients in this subgroup were seven through 16 years of age, at baseline, walked no more than 80% of predicted 6-minute walk distance compared to healthy boys matched for age and height, but had the ability to walk at least 150 meters during the 6-minute walk test, and had used systemic corticosteroids for a minimum of six months prior to start of treatment. The results of this subgroup analysis are shown in the graph and the table below.

Mean change in 6-minute walk distance (6MWD) in Phase 2b subgroup based on enrollment criteria for confirmatory Phase 3 clinical trial (corrected ITT analysis)



Summary of change from baseline to week 48	Treatment arm: Placebo N=31	Treatment arm: Ataluren 10, 10, 20 dose N=30
Mean (standard deviation), meters	-62.2 (84.9)	-12.3 (69.4)
Mean difference from placebo, meters		49.9
Nominal p-value (vs. placebo)		0.0096

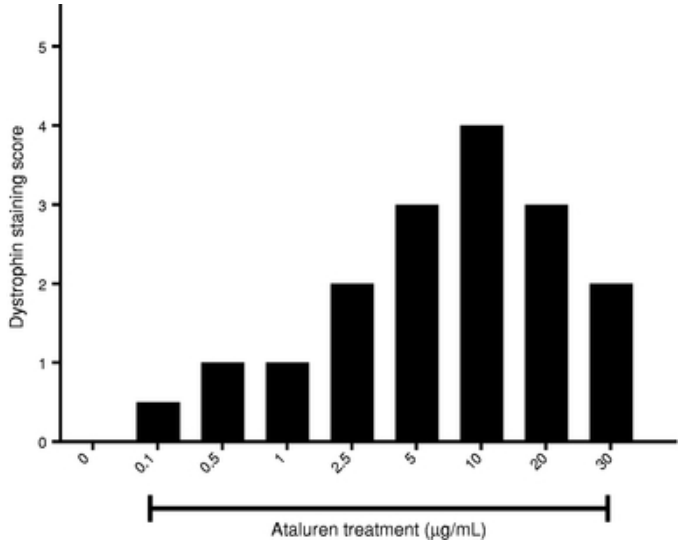
In this subgroup analysis, the difference between the 10, 10, 20 dose of ataluren and placebo in mean change in 6-minute walk distance over 48 weeks was 49.9 meters (nominal p = 0.0096). Because all patients in this subgroup were receiving corticosteroids, the variability was reduced compared to the mixed population in the Phase 2b trial of corticosteroid users and non-users.

Dose-response curve. In our Phase 2b clinical trial, although the 10, 10, 20 dose of ataluren showed clinically meaningful improvements over placebo, the 20, 20, 40 dose of ataluren generally showed little or no difference from placebo. Based on our current understanding of the ribosome's structure, we believe that the 10, 10, 20 dose of ataluren associates with a particular site on the ribosome that allows the ribosome to read through a premature stop signal. We believe that this allows the ribosome to make the full-length, functional dystrophin protein and modulate the defect in muscular dystrophy. At higher doses, such as the 20, 20, 40 dose, we believe that ataluren may also interact with a second site on the ribosome that interferes with the ability of the ribosome to read through the premature stop signal. Therefore, we believe that at higher doses ataluren no longer enables the ribosome to make the full-length, functional dystrophin protein.

The results of our Phase 2b clinical trial are consistent with a bell-shaped dose-response in which the response to treatment initially increases with higher drug concentrations and then subsequently decreases at even higher drug concentrations. We also observed a bell-shaped dose-response to ataluren in non-clinical studies involving mouse and human cellular models of nmDMD and a mouse cellular model of the genetic disorder Hurler's Disease and in two extension trials of ataluren that we conducted.

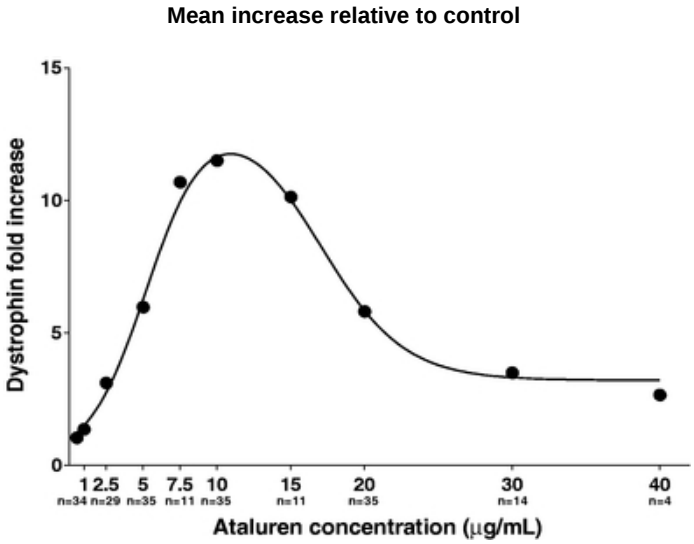
Mouse model of nmDMD. In a mouse model of nmDMD, ataluren increased dystrophin production in muscle cells grown in the laboratory, referred to as myotubes, with maximal activity at a concentration of 10 micrograms per milliliter, or mg/ml. In this study, we observed decreased dystrophin production at concentrations of ataluren ranging from 20 mg/ml to 30 mg/ml, indicating a bell-shaped dose-response curve. We assessed dystrophin production based on staining of muscle samples with antibodies as observed under a microscope. The figure below shows the results from this mouse model.

Effect of ataluren on dystrophin staining scores in myotubes isolated from nmDMD mice



Myotubes from nmDMD patients. In non-clinical studies with human myotubes grown from 35 different nmDMD patients, ataluren also exhibited a bell-shaped dose-response curve with maximal dystrophin staining observed at 10 mg/ml of ataluren. At concentrations above 10 mg/ml, there was diminishing response to treatment. The figure below shows the results from these non-clinical studies. In this figure, the values on the vertical axis represent changes in dystrophin expression relative to untreated myotube cultures from patients with nmDMD.

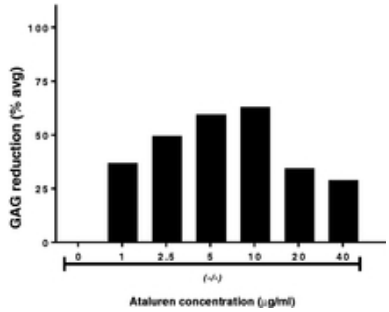
***In vitro* dystrophin expression concentration-response in cultured myotubes from nmDMD patients**



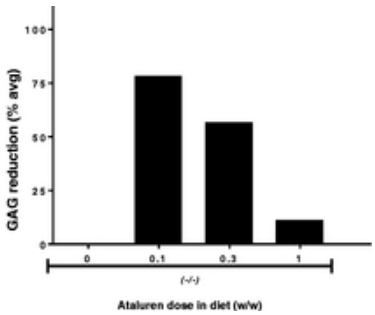
Mouse model of nonsense mutation Hurler's disease. In addition, ataluren also showed a bell-shaped dose-response in two experiments in a mouse model that we developed for the genetic disorder Hurler's disease in which the mice harbor a nonsense mutation. In cells taken both from the mouse model exposed

to increasing concentrations of ataluren, shown in the first figure below, and in spleen samples from mice given increasing doses of ataluren, shown in the second figure below, we observed a bell-shaped dose-response in the reduction in the levels of glycosaminoglycans, or GAGs, elevated levels of which are a hallmark of Hurler's disease.

In vitro **mouse cells**

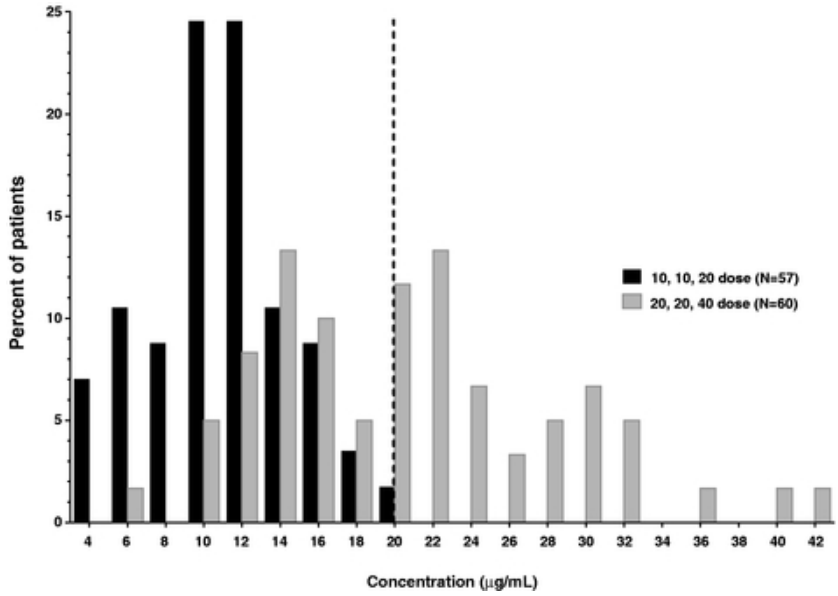


In vivo **mouse spleen**



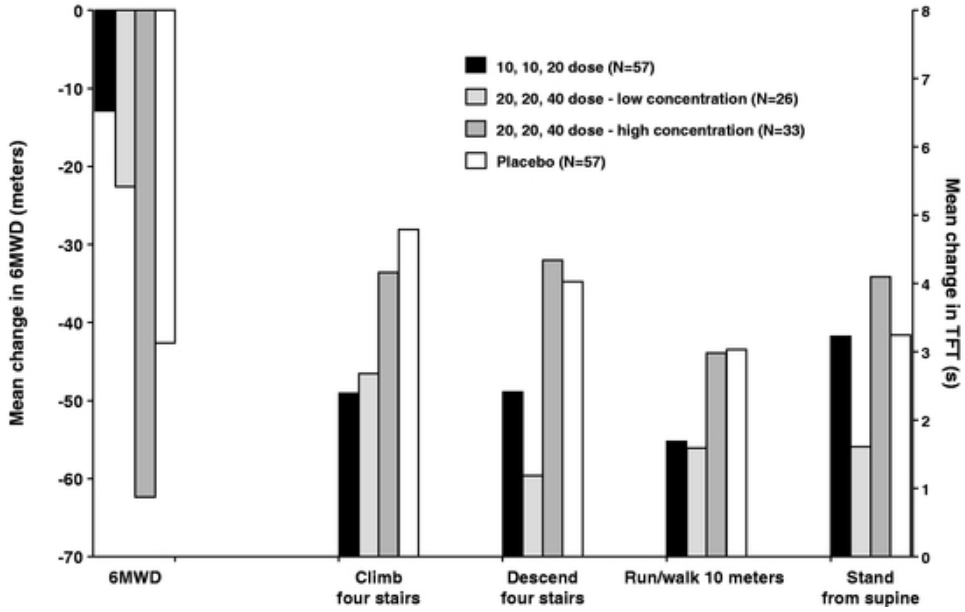
Clinical trial data. To gather additional evidence of a bell-shaped dose-response curve, we analyzed the data from our clinical trials based on ataluren plasma concentration. In our Phase 2b clinical trial, we measured ataluren plasma concentrations prior to the morning dose, or C_{0h}, and two hours after the morning dose, or C_{2h}, at each visit. All patients receiving the 10, 10, 20 dose of ataluren had a maximal mean plasma concentration of less than 20 mg/ml. Approximately 40% of patients receiving the 20, 20, 40 dose had mean plasma concentrations in a low concentration range of less than 20 mg/ml, and approximately 60% of these patients had mean plasma concentrations in a high concentration range of 20 mg/ml or greater. The figure below shows mean C_{2h} ataluren data from our Phase 2b clinical trial.

Mean 2-hour ataluren plasma concentrations



In our Phase 2b clinical trial, we also analyzed 6-minute walk distance and timed function tests by mean C_{0h} and C_{2h} across all visits. Patients who received the 20, 20, 40 dose of ataluren and had mean plasma concentrations in a low concentration range of less than 20 mg/ml had better results in 6-minute walk distance and time function tests than patients who received the 20, 20, 40 dose of ataluren but had mean plasma concentrations in a high concentration range of 20 mg/ml or greater. The figure below shows these results.

Mean change in 6-minute walk distance (6MWD) and timed function tests (TFT(s)) by concentration



We performed similar concentration-response analyses, based on $C_{0h,}$ in our Phase 2a and Phase 2b open label extension trials in which only the 20, 20, 40 dose of ataluren was evaluated. In both trials, patients with mean plasma concentrations in a low concentration range of less than 20 mg/ml had better results in 6-minute walk distance and timed function tests than patients with mean plasma concentrations in a high concentration range of 20 mg/ml or greater. This indicates that the plasma concentration range associated with the 10, 10, 20 dose is the active concentration range of ataluren.

Secondary endpoints. Our analyses of the data from the secondary efficacy endpoints of this Phase 2b clinical trial are summarized below. There was little or no prior experience with several of these secondary endpoints in Duchenne muscular dystrophy therapeutic trials. In addition, this trial was powered based upon the primary endpoint, 6-minute walk distance, and not to detect statistically significant differences in these secondary endpoints. However, patients in the 10, 10, 20 ataluren dose group trended better than the placebo group in several of these secondary endpoints.

- *Timed tests of muscle function.* Patients treated with ataluren showed less decline in muscle function over 48 weeks, as evidenced by smaller increases in the times to climb four stairs, descend four stairs and run/walk 10 meters, relative to placebo. These trends were more prominent with the 10, 10, 20 dose of ataluren and exceeded the clinically meaningful threshold of 1.5 seconds for stair-climbing and stair-descending in the ITT analysis and for running/walking in the corrected ITT analysis. In a supine to stand test, we did not observe any difference between ataluren and placebo.

- *Muscle strength.* We performed myometric evaluations, in which muscle strength is measured in knee flexion, knee extension, elbow flexion, elbow extension and shoulder abduction. Over 48 weeks, patients treated with ataluren generally showed slightly less decline in muscle strength, as evidenced by smaller decreases in most myometry parameters, relative to placebo. These trends were more prominent with the 10, 10, 20 dose of ataluren, although differences were below a threshold considered to be clinically meaningful.

- *Frequency of accidental falls.* The frequency of falls was measured based on a diary kept by patients/caregivers. Trial results showed trends in reductions in accidental falling for ataluren compared to placebo. Accidental falls are a major concern of patients and their families, since they can lead to fractures and, in some cases, loss of ambulation.

- *Patient-reported health related quality of life and treatment satisfaction.* We observed positive trends favoring the 10, 10, 20 dose of ataluren compared to placebo in the patient reported physical functioning aspect of the health related quality of life measurement, although differences were below a threshold considered to be clinically meaningful.

- *At-home activity as measured by pedometry.* In an assessment of time spent at different activity levels in daily life, the largest differences between ataluren and placebo in mean changes at week 48 were observed with the 10, 10, 20 dose of ataluren, which showed trends toward less time spent at no activity and more time spent at medium activity. We performed this assessment on the basis of the number of steps taken per minute as measured by a pedometer worn on the ankle. In conjunction with this step activity monitoring, patients receiving the 10, 10, 20 dose of ataluren showed trends toward less increase in wheelchair use over 48 weeks as compared to placebo.

- *Biceps muscle dystrophin expression.* Because muscular dystrophy is caused by the absence of the dystrophin protein, we sought to collect quantitative data with respect to muscle dystrophin expression as we had observed in our Phase 2a clinical trial described below. However, we were unsuccessful in doing so, in part, because a majority of muscle biopsy samples that we collected were compromised, which precluded meaningful interpretation of the data. In addition, we have since concluded that a sensitive and reliable method is not currently available for quantifying dystrophin at the low levels seen in patients with Duschenne muscular dystrophy and that muscle sampling is problematic because of the variation in dystrophin levels within either the same muscle or between different muscles.

- *Other secondary endpoints.* Changes in other secondary efficacy endpoints were generally small, and we did not observe any clear differentiation between ataluren and placebo.

Safety and tolerability. Ataluren was generally well tolerated at both dose levels in our Phase 2b clinical trial. There were no study discontinuations due to adverse events and no ataluren-related serious adverse events were reported. Most treatment-emergent adverse events were mild or moderate in severity. Investigators' attributions of drug-related adverse effects were generally similar across the placebo and ataluren arms. An overview of adverse events in this trial is shown in the table below.

Overview of treatment-emergent adverse events in Phase 2b clinical trial (as-treated population)

Parameter	Treatment arm Placebo N=57	Ataluren 10, 10, 20 dose N=57	Ataluren 20, 20, 40 dose N=60	All patients N=174
Patients with ³1 adverse event	56 (98.2)%	55 (96.5)%	57 (95.0)%	168 (96.6)%
Adverse events by severity				
Grade 1 (mild)	21 (36.8)%	16 (28.1)%	20 (33.3)%	57 (32.8)%
Grade 2 (moderate)	26 (45.6)%	31 (54.4)%	27 (45.0)%	84 (48.3)%
Grade 3 (severe)	9 (15.8)%	8 (14.0)%	10 (16.7)%	27 (15.5)%
Grade 4 (life-threatening)	—	—	—	—
Adverse events by relatedness				
Unrelated	14 (24.6)%	8 (14.0)%	11 (18.3)%	33 (19.0)%
Unlikely	16 (28.1)%	17 (29.8)%	13 (21.7)%	46 (26.4)%
Possible	20 (35.1)%	25 (43.9)%	29 (48.3)%	74 (42.5)%
Probable	6 (10.5)%	5 (8.8)%	4 (6.7)%	15 (8.6)%
Discontinuations due to adverse events	—	—	—	—
Serious adverse events	3 (5.3)%	2 (3.5)%	2 (3.3)%	7 (4.0)%
Deaths	—	—	—	—

Open label continuation trials of ataluren for nmDMD

We are currently conducting two open label continuation trials to evaluate the safety and tolerability of ataluren in patients with nmDMD who previously participated in one of our other clinical trials. We are conducting one of these continuation trials in the United States and the other in countries outside the United States. We plan to enroll up to 122 patients in the U.S. trial and approximately 96 patients in the other trial. We initiated the U.S. trial in November 2010 and the other trial in May 2012. As of February 28, 2013, we had enrolled 107 patients in the U.S. trial and 61 patients in the other trial. Patients in these trials receive the 10, 10, 20 dose of ataluren at morning, midday and evening. Study assessments are performed at clinic visits every 12 weeks. As of February 1, 2013, available data from these continuation trials indicated no change in the safety profile for ataluren in patients with nmDMD.

Phase 2a clinical trial of ataluren for nmDMD

In October 2007, we announced the results of an open label Phase 2a clinical trial evaluating ataluren in 38 patients with nmDMD as confirmed by gene sequencing. We conducted this trial at three academic centers in the United States.

The primary objective of this trial was to obtain indications of pharmacological activity. The primary efficacy endpoint in this trial was the change from baseline measurement of dystrophin levels in a muscle in the foot known as the extensor digitorum brevis, or EDB. In this trial, the entire EDB muscle was removed from one foot prior to treatment and the entire EDB muscle was removed from the other foot after treatment. An increase from baseline in study participants' dystrophin levels in the EDB muscle biopsy indicates suppression of the nonsense mutation. We evaluated dystrophin protein levels using immuno-fluorescent staining. Secondary endpoints of the trial included serum CK levels, changes in muscle

strength, time taken to perform specified functions such as walking and climbing stairs and compliance with ataluren treatment. The trial also assessed dose-response and the safety and pharmacokinetic profiles of ataluren.

Patients enrolled in this trial were at least five years of age, were diagnosed with nonsense mutation Duchenne muscular dystrophy, had increased levels of serum CK and had absent or diminished dystrophin protein on muscle biopsy.

Participants in the trial were divided into three groups, with all participants in each group receiving ataluren treatment for 28 days. The first group comprised the first six participants in the trial, who received a dosing regimen of ataluren consisting of 4 mg/kg in the morning, 4 mg/kg at midday and 8 mg/kg in the evening. The second group comprised the next 20 participants in the trial, who received the 10, 10, 20 dose of ataluren. The third group comprised the final 12 participants in the trial, who received the 20, 20, 40 dose of ataluren.

We tested the effects of ataluren on trial participants at the end of the 28-day treatment period and conducted a follow-up assessment four weeks after the last dose administration.

In this trial, ataluren induced a mean 11.1% increase in muscle dystrophin expression over the 28 days of treatment, with 23 of the 38 patients (61%) showing an increase from baseline. We observed serum CK reductions in 35 of the 38 patients (92%) at the end of treatment. With cessation of ataluren treatment, mean serum CK concentrations reverted toward baseline. Changes in myometry scores and timed function tests were small and not statistically significant with 28 days of ataluren treatment. Anecdotal reports from the parents and teachers of several boys noted evidence of greater activity, increased endurance and less fatigue during ataluren administration. Pharmacokinetic results from this trial indicated that both the 10, 10, 20 dose and the 20, 20, 40 dose regimens achieved plasma concentrations of ataluren that were predicted to have a therapeutic effect, based on preclinical data. The 4, 4, 8 dose regimen did not consistently achieve these levels, and as a result we did not include this dosing regimen in our subsequent Phase 2b trial.

We observed mild treatment emergent adverse events of transient headache and gastrointestinal complaints, which appeared consistent with background symptoms commonly observed in clinical trials. There were no clearly dose dependent increases in frequency or severity of adverse events. No drug related serious adverse events were reported. Patients received more than 99% of the planned ataluren doses, and no patient discontinued ataluren due to an adverse event.

Cystic fibrosis

Cystic fibrosis is among the most common life-threatening genetic disorders worldwide. According to the Cystic Fibrosis Foundation, cystic fibrosis occurs in approximately one of every 3,500 live births in the United States, with approximately 1,000 new cases diagnosed each year in the United States. Commercially available genetic testing can determine if a patient's cystic fibrosis is caused by a nonsense mutation. The Cystic Fibrosis Foundation estimates that approximately 83% of the active patients in their National Patient Registry have been genotyped. According to the Cystic Fibrosis Foundation, the disease affects approximately 30,000 adults and children in the United States. Based on data from the Journal of Cystic Fibrosis, we believe the disease affects between approximately 37,000 and 42,000 adults and children in the European Union. Based on information from the Cystic Fibrosis Foundation, we estimate that nonsense mutations are the cause of cystic fibrosis in approximately 10% of patients, or approximately 3,000 patients in the United States and approximately 3,700 to 4,200 patients in the European Union.

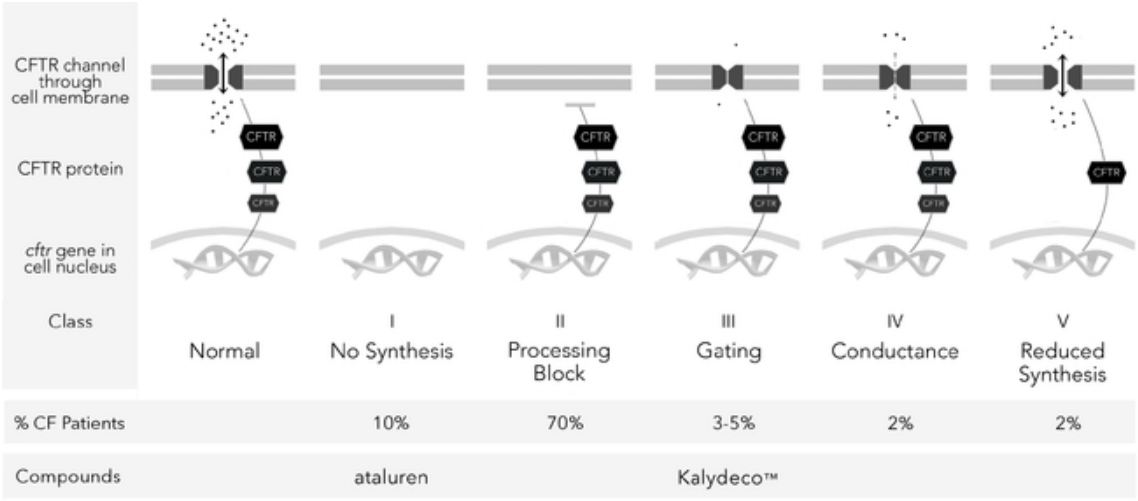
Cystic fibrosis is caused by defects in a single gene known as the cystic fibrosis transmembrane conductance regulator, or CFTR. The CFTR gene encodes the CFTR protein, which is used by the body to

transport chloride across cell membranes. Genetic mutations that result in the loss of function of the CFTR protein cause the body to produce abnormally thick and sticky mucus that clogs multiple organs, including the lungs, pancreas and liver. In particular, the absence or very low levels of CFTR leads to progressive loss of lung function, potentially life-threatening lung infections, permanent pancreatic damage and malnutrition because digestive enzymes from the pancreas do not reach the intestines to help break down and absorb food. Because patients with cystic fibrosis have malabsorption and a high calorie expenditure for breathing, their body weights are often low.

Complications from lung infections are the primary cause of death from cystic fibrosis. From as early as four months of age, patients with cystic fibrosis may begin to develop airway obstruction and inflammation. Over time, most patients develop chronic bacterial infections in the airways, resulting in repeated episodes of pneumonia. Ultimately, progressive lung dysfunction leads to respiratory failure and death. According to the Cystic Fibrosis Foundation's National Patient 2011 Registry, the average age of death for cystic fibrosis patients is in their mid-thirties.

Mutations causing cystic fibrosis are categorized in five different classes, Class I through Class V, as represented in the figure below. Class I consists of nonsense mutations and is the most severe because there is absence of CFTR production and no CFTR on the surface of the lung cells. Patients from six to 18 years of age with two Class I mutations, one on each of a pair of genes, have on average 10% lower forced expiratory volume in one second, or FEV_1, measures than patients with two Class II mutations. FEV_1 is a measure of the volume of air that has been exhaled at the end of the first second of forced expiration. Ataluren targets Class I mutations. Class II mutations are targeted by corrector drugs, which promote the production or movement of CFTR protein from within the cell to the cell surface. In contrast, the milder mutations, Class III, IV and V, are targeted by potentiator drugs, which enhance the effect of abnormal CFTR that is already present on the cell surface. The FDA recently approved Kalydeco, developed by Vertex Pharmaceuticals, as a treatment for patients with a Class III mutation known as G551D that occurs in approximately 3% to 5% of cystic fibrosis patients. Ataluren and Kalydeco have not been tested in combination in any clinical trials.

Different types of genetic mutations cause cystic fibrosis



There is currently no marketed therapy approved to correct defective CFTR production and function in patients with nmCF. For nmCF patients, available treatments do not address the underlying cause of the disease and are designed only to alleviate the symptoms of the disease. These treatments include chest physical therapy to clear the thick mucus from the lungs, antibiotics to treat lung infections and a mucus-thinning drug designed to reduce the number of lung infections and improve lung function. In addition, the majority of cystic fibrosis patients take pancreatic enzyme supplements to assist with food absorption in digestion.

Planned Phase 3 clinical trial of ataluren for nmCF

We are planning a multicenter, randomized, double-blind, placebo controlled Phase 3 clinical trial to evaluate the efficacy and safety of ataluren in approximately 210 patients with cystic fibrosis caused by a nonsense mutation as confirmed by gene sequencing. We plan to initiate this trial in the second half of 2013, subject to the conclusion of our ongoing discussions with regulatory authorities regarding our proposed trial design. We expect that the primary objective of this trial will be to evaluate the effect of ataluren on pulmonary function relative to placebo based on a primary efficacy endpoint of relative change in percent of predicted FEV_1. Percent of predicted FEV_1, or %-predicted FEV_1, is based on a comparison to healthy individuals matched for age, height and gender. We expect that secondary efficacy endpoints in the trial will include the following:

- pulmonary exacerbation rate, based on specified signs and symptoms; and
- other pulmonary function measures as assessed by lung capacity and expiratory flow.

We expect to require that patients in this trial be at least six years of age and have %-predicted FEV_1 within a specified range, sweat chloride in excess of a specified level as evidence of the severity of the disease and documentation of a nonsense mutation in at least one copy of the CFTR gene. We expect to exclude patients from the trial if they are receiving chronic inhaled aminoglycoside antibiotics, have any change in treatment or prophylaxis for cystic fibrosis related conditions within four weeks prior to start of study treatment, have recently been treated with intravenous antibiotics or have major complications of lung disease. We expect to perform study assessments of FEV_1 at clinic visits every eight weeks and that patients will undergo 48 weeks of blinded treatment prior to the final analysis.

We plan to stratify patients based on age, screening %-predicted FEV_1 and chronic use of inhaled antibiotics. We plan to randomize patients in a 1:1 ratio to receive either placebo or ataluren at a dosing regimen of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg. At the completion of blinded treatment, we plan to make an open label extension trial available to patients who successfully complete the double-blind trial and are not in countries where ataluren is commercially available at that time.

Regulatory status and strategy for nmCF

EMA. We have received scientific advice from the EMA regarding the possibility of submitting an MAA for conditional approval of ataluren for the treatment of nmCF and the protocol design of a post-approval confirmatory trial. The EMA recognized that there is an unmet medical need and advised us that it would consider an MAA for conditional approval of ataluren for patients with nmCF. If we submit an MAA for this condition, approval will depend on the EMA's assessment of the relative risks and benefits of conditional approval and our ability to provide comprehensive clinical data from a post-approval confirmatory trial. The EMA has informed us that the benefit from ataluren that we observed in our completed Phase 3 clinical trial would have been more demonstrative if we had used absolute change in %-predicted FEV_1

rather than relative change in %-predicted FEV_1 as the primary efficacy endpoint, although the EMA also acknowledged that relative change in %-predicted FEV_1 can nonetheless be considered an acceptable primary endpoint in our planned Phase 3 clinical trial. We may not be able to demonstrate the required relative risk-benefit profile or the likelihood that we can provide the required confirmatory trial data for ataluren for this indication. There is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of nmCF.

In particular, the EMA has advised us that we may need to address the following additional matters in our MAA:

- the clinical relevance of the relative change in %-predicted FEV_1 that we observed in our completed Phase 3 clinical trial after taking into account all possible biases and confounders;
- consideration of alternatives to explain the lack of statistical significance in relative change in %-predicted FEV_1 in our Phase 3 clinical trial other than the plausible explanation that the inhaled antibiotic tobramycin interfered with ataluren's mechanism of action;
- lack of clear support from secondary or tertiary endpoints in our completed Phase 3 clinical trial;
- the imbalance in the baseline %-predicted FEV_1 in favor of ataluren;
- the evolution of the relative change in %-predicted FEV_1 over 48 weeks in the placebo group being worse than expected;
- the lack of analysis of patients based on body weight and height; and
- the statistical analysis that we employed.

FDA. We met with the FDA in July 2012 to discuss the results of our completed Phase 3 clinical trial of ataluren for the treatment of nmCF. The FDA indicated that in its view the data from our completed Phase 3 trial and other data from our development program in cystic fibrosis do not by themselves support an NDA submission. Consequently, the FDA informed us that additional clinical data would be required to establish the evidence required to support eventual filing of an NDA for the use of ataluren to treat nmCF. We have begun discussions with the FDA regarding the clinical development design options which would have the potential to support an NDA. Our goal in these continuing discussions is to achieve consensus between the EMA and the FDA that a single placebo controlled Phase 3 trial can serve as the basis for full approval of ataluren to treat nmCF in both the European Union and the United States.

Completed Phase 3 clinical trial of ataluren for nmCF

In June 2012, we announced the results of a multicenter, international, randomized, double-blind, placebo controlled Phase 3 clinical trial assessing the effects of ataluren in 238 patients with cystic fibrosis caused by a nonsense mutation as confirmed by gene sequencing. The primary objective of this trial was to evaluate the effect of ataluren on pulmonary function relative to placebo. The primary efficacy endpoint was relative change in %-predicted FEV_1. The trial assessed pulmonary exacerbation rate as a secondary efficacy endpoint.

Patients enrolled in this trial were at least six years of age, weighed at least 16 kilograms and had a %-predicted FEV_1 between 40% and 90%, sweat chloride in excess of a specified level, a minimum level of resting oxygen saturation in the blood and documentation of a nonsense mutation in at least one copy of the CFTR gene. We excluded patients from the trial if they had any change in treatment or prophylaxis for cystic fibrosis related conditions within four weeks prior to start of study treatment, had evidence of pulmonary exacerbation or acute upper or lower respiratory tract infection, were treated with intravenous antibiotics or had major complications of lung disease.

We stratified patients in this trial based on age, baseline %-predicted FEV_1 and chronic use of inhaled antibiotics. Patients were randomized in a 1:1 ratio to receive placebo or ataluren at a dosing regimen of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg. The trial protocol specified a clinic visit every eight weeks to assess FEV_1. The treatment duration was 48 weeks.

We designed the trial to detect a mean relative change in %-predicted FEV_1 from baseline to end of treatment at week 48 that was at least 6% greater in the ataluren arm than in the placebo arm. Of the 238 total patients, 120 patients received ataluren and 118 patients received placebo, with 34 patients withdrawing prematurely, including 20 patients on ataluren and 14 patients on placebo. One patient completed 48 weeks of blinded therapy but did not have evaluable FEV_1 data at week 48. This resulted in 203 patients completing the 48-week treatment period with FEV_1 data available at week 48. As specified in the trial protocol, the ITT population included all randomized patients who had FEV_1 data available at baseline and at least one post-baseline visit, resulting in 116 patients on ataluren and 116 patients on placebo being included in the ITT population.

The percent of the initial total value that was changed is referred to as relative change. The change in percentage that is representative of the difference alone is referred to as absolute change. For example, when 50% changes to 55%, the result is a 10% relative change and a 5% absolute change. The results from this trial are shown in the table below. The table shows information about relative change, which was the primary analysis, and absolute change in %-predicted FEV_1 from baseline to week 48.

Change in %-predicted FEV_1 from baseline to week 48 (ITT population)

	Placebo N=116	Ataluren 10, 10, 20 dose N=116
Relative change in %-predicted FEV_1 at week 48		
Mean (standard deviation)	-5.5% (12.56)	-2.5% (13.25)
Mean difference from placebo		3.0%
p-value		0.124
Relative change in %-predicted FEV_1 averaged over 48 weeks		
Mean	-4.3%	-1.8%
Mean difference from placebo		2.5%
p-value		0.0478
Absolute change in %-predicted FEV_1 at week 48		
Mean (standard deviation)	-3.1% (7.39)	-1.3% (8.50)
Mean difference from placebo		1.8%
p-value		0.136

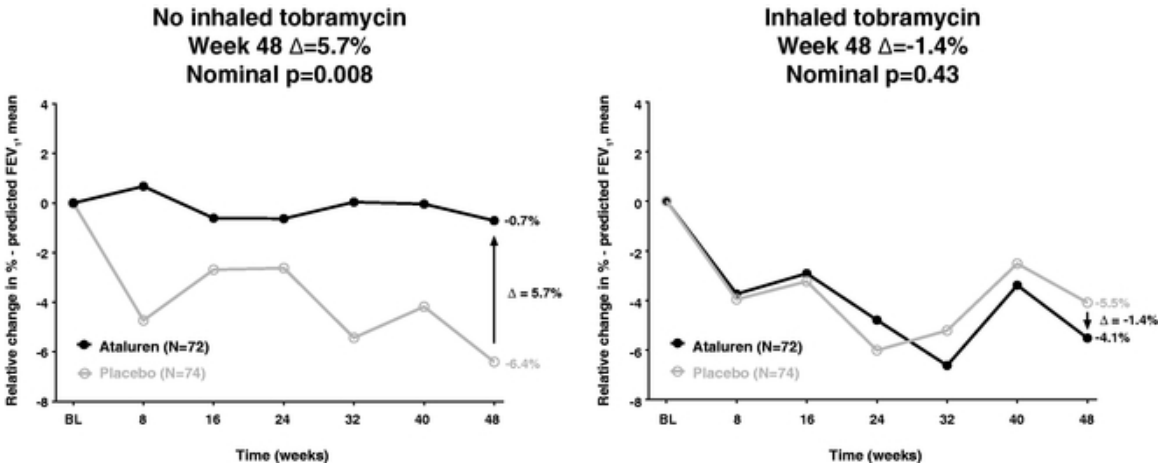
The primary analysis of relative change in %-predicted FEV_1 in this trial showed a 3.0% difference (2.5% decrease on ataluren, 5.5% decrease on placebo) at week 48 favoring ataluren (p=0.124), which was not statistically significant. An analysis of relative change in %-predicted FEV_1 based on the average treatment effect across all post-baseline visits showed a statistically significant difference of 2.5% favoring ataluren compared to placebo (1.8% decrease on ataluren, 4.3% decrease on placebo; p=0.0478). The analysis of treatment effect across all visits was part of the pre-specified statistical model for this trial and has served as the primary analysis of FEV_1 data in other cystic fibrosis therapeutic trials conducted by other companies. The analysis of absolute change in %-predicted FEV_1 at week 48 showed a 1.8% difference (1.3% decrease on ataluren, 3.1% decrease on placebo; p =0.136).

Subgroup analysis of patients not receiving inhaled antibiotics. As described above, we pre-specified three stratification factors in this trial: age, baseline FEV_1 and chronic use of inhaled antibiotics. In this trial, there was a statistically significant interaction (nominal p=0.0108) between treatment and chronic inhaled antibiotic use. As discussed in more detail below, we believe that the inhaled antibiotic tobramycin interfered with ataluren's mechanism of action. The interactions between treatment and age and between treatment and baseline %-predicted FEV_1 were not significant.

For the subgroup of patients not receiving chronic inhaled antibiotics, the difference in mean relative changes from baseline in %-predicted FEV_1 at week 48 was 6.7% favoring ataluren (nominal p=0.013). The average treatment effect across all post-baseline visits was 5.4% (nominal p=0.0014). For absolute change in %-predicted FEV_1, the average treatment effect across all post-baseline visits was 3.1% (nominal p=0.003). In contrast, patients that received chronic inhaled antibiotics and ataluren did not exhibit a difference compared to patients that received chronic inhaled antibiotics and placebo.

Approximately 37% of patients in the trial were receiving the chronic inhaled antibiotic tobramycin, and approximately 45% of patients were receiving no chronic inhaled antibiotic. Other chronic inhaled antibiotics that patients received were colistin or aztreonam. We performed analyses comparing patients not receiving chronic inhaled tobramycin to patients receiving chronic inhaled tobramycin. The results for patients not receiving chronic inhaled tobramycin and patients receiving chronic inhaled tobramycin are depicted in the following graphs.

Mean relative change in %-predicted FEV_1 at week 48 by baseline chronic inhaled tobramycin use



In patients not receiving chronic inhaled tobramycin, the difference in mean relative change from baseline in %-predicted FEV_1 at week 48 was 5.7% favoring ataluren (nominal p=0.008), consistent with the targeted treatment effect size. Patients receiving chronic inhaled tobramycin did not show a benefit for ataluren compared to placebo in %-predicted FEV_1. In contrast, the treatment effect was similar in patients receiving colistin or aztreonam compared to patients not receiving colistin or aztreonam.

Both tobramycin and ataluren act through modulation of the ribosomal machinery. We believe that the binding of tobramycin to the ribosome may interfere with ataluren's mechanism of action. We explored this hypothesis in a functional cell-based translation assay. In this experiment, ataluren-induced read-through of premature stop codons was diminished when the cells were exposed to ataluren together with tobramycin

or gentamicin, but not when ataluren was administered together with colistin or aztreonam, both of which are non-aminoglycosides.

Pulmonary exacerbation rate. The secondary endpoint in this trial was pulmonary exacerbation rate, which is a measure of frequency of lung infections related to cystic fibrosis. FEV_1 and pulmonary exacerbation rate are the two most clinically important outcome measures in cystic fibrosis trials. In the ITT population, we observed a 23% lower pulmonary exacerbation rate in patients receiving the 10, 10, 20 dose of ataluren than placebo ($p = 0.099$). This result was not statistically significant. However, we also saw the tobramycin subgroup effect in this endpoint. Patients not receiving chronic inhaled tobramycin had a 41% lower pulmonary exacerbation rate on ataluren than placebo (nominal $p=0.005$). Patients receiving chronic inhaled torbramycin did not show a benefit in pulmonary exacerbation rate on ataluren as compared to placebo.

Tertiary Endpoints. In this trial, we assessed CFTR function by nasal transepithelial difference, or TEPD, and sweat chloride concentration as tertiary endpoints. TEPD is assessed by means of a standardized, though complex, minimally invasive procedure. In the procedure, a small plastic catheter is used to assess electrical differences across the outer cell membrane of nasal mucosa cells in the nostril. Nasal TEPD is physiologically meaningful because nasal mucosa closely reflects CFTR activity in the lung epithelium. Because of the role of the CFTR protein in transporting chloride across cell membranes and because of the absence of this protein in cystic fibrosis patients, these patients have an abnormal TEPD chloride conductance. Sweat chloride concentration is a commonly used test to diagnose cystic fibrosis and is a measurement of CFTR activity in the sweat gland.

A number of clinical trials for CFTR restoration therapies have used sweat chloride concentration and nasal TEPD as pharmacodynamic endpoints. However, these two endpoints can exhibit varying results, likely because of differences in CFTR regulation and function in the sweat glands as compared to the nasal or lung mucosa, or variation in tissue penetration of different drugs.

Nasal TEPD results were positive in our prior Phase 2 clinical trials discussed below, but sweat chloride testing was not positive in either Phase 2 clinical trial or in our Phase 3 clinical trial. In contrast with our Phase 2 clinical trials, in which we assessed TEPD at a small number of experienced sites, in the Phase 3 trial, TEPD assessments were performed at all centers. This trial was the first time most centers had performed TEPD assessments. In this trial, TEPD results showed high variability and an unexpectedly high response rate on placebo.

The other tertiary endpoints in this trial were hourly cough rate, respiratory domain score from a questionnaire, inflammatory markers and lung computed tomography. Differences between ataluren and placebo for each of these endpoints were small and not statistically significant.

Safety and tolerability. Ataluren was generally well tolerated in this clinical trial, and there were generally similar adverse event profiles in patients treated with ataluren and patients treated with placebo. Most serious adverse events were cystic fibrosis pulmonary exacerbations unrelated to study drug treatment. Most treatment-emergent adverse events were mild or moderate in severity. Investigators' attributions of severity and drug-relatedness were generally similar across the placebo and ataluren arms. The most common adverse events were typical of cystic fibrosis. Eleven patients prematurely discontinued treatment because of adverse events, including eight in the ataluren arm and three in the placebo arm.

There were 19 patients with at least one treatment-emergent renal adverse event, including 15 patients receiving ataluren and 4 patients receiving placebo. In the ataluren arm, five adverse events that involved the renal system led to discontinuation. As compared to the placebo group, the ataluren treatment arm also had a higher incidence of adverse events of creatinine elevations, which can be an indication of

impaired kidney function. These adverse events of creatinine elevations were generally mild and transient. In the ataluren treatment arm, clinically meaningful creatinine elevations of grade 3 or grade 4 were reported in conjunction with cystic fibrosis pulmonary exacerbations. These creatinine elevations were associated with concomitant treatment with antibiotics associated with impaired kidney functions, such as aminoglycosides or vancomycin. This led to the subsequent prohibition of concomitant use of ataluren and these potentially nephrotoxic antibiotics, which was successful in addressing this issue. The incidence of new-onset kidney stones was similar in both arms, with five patients in the ataluren arm and four patients in the placebo arm.

An overview of adverse events in this trial is shown in the table below.

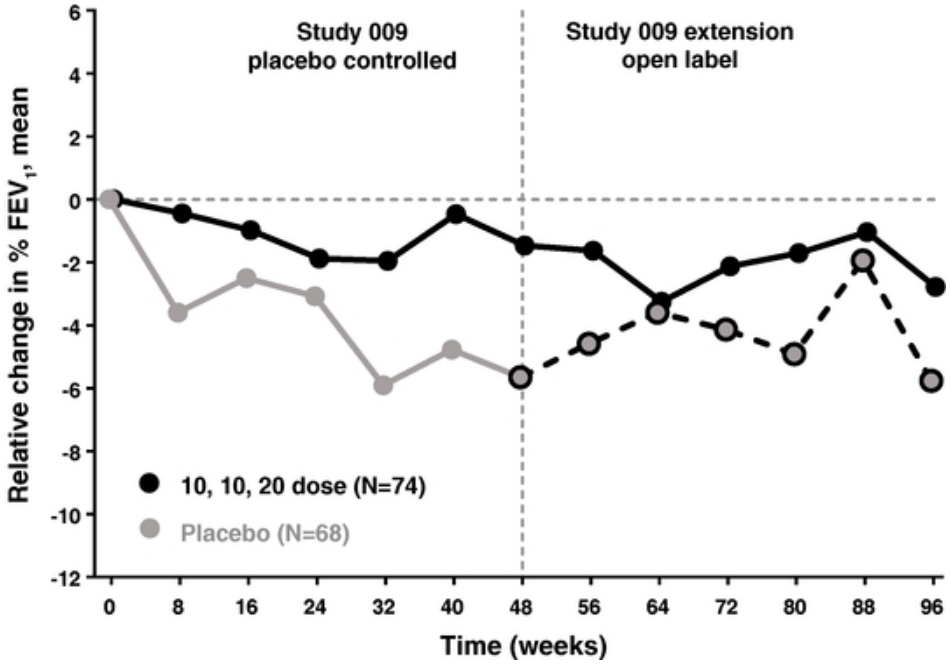
Overview of treatment-emergent adverse events in Phase 3 clinical trial (as-treated population)

	Treatment arm		
Parameter	**Placebo N=118**	**Ataluren N=120**	**All patients N=238**
Patients with ³1 adverse event	115 (97.5)%	118 (98.3)%	233 (97.9)%
Adverse events by severity			
Grade 1 (mild)	20 (16.9)%	18 (15.0)%	38 (16.0)%
Grade 2 (moderate)	65 (55.1)%	81 (67.5)%	146 (61.3)%
Grade 3 (severe)	30 (25.4)%	19 (15.8)%	49 (20.6)%
Grade 4 (life-threatening)	—	—	—
Adverse events by relatedness			
Unrelated	42 (35.6)%	30 (25.0)%	72 (30.3)%
Unlikely	31 (26.3)%	39 (32.5)%	70 (29.4)%
Possible	35 (29.7)%	34 (28.3)%	69 (29.0)%
Probable	7 (5.9)%	15 (12.5)%	22 (9.2)%
Discontinuations due to adverse events	3 (2.5)%	8 (6.7)%	11 (4.6)%
Serious adverse events	48 (40.7)%	45 (37.5)%	93 (39.1)%
Deaths	—	—	—

Open label extension trial of ataluren for treatment of nmCF

We are currently conducting an open label, extension trial that is providing additional safety information for the long term administration of ataluren in patients with cystic fibrosis who successfully completed 48 weeks of treatment in our completed Phase 3 clinical trial. In addition, this trial is designed to provide supportive long-term efficacy information to better understand the long-term effects of ataluren on pulmonary function and pulmonary exacerbations. This trial enrolled 191 of the patients who completed the double-blind Phase 3 clinical trial described above. Patients in this trial receive the 10, 10, 20 dose of ataluren for a 96 week treatment period. We are performing study assessments at clinic visits every eight weeks. Currently available data on FEV_1 in patients who have completed a total of 96 weeks of treatment are shown in the figure below. In patients who have received ataluren since the beginning of the Phase 2b clinical trial, FEV_1 has been generally maintained over the course of 96 weeks. In patients who transitioned from placebo to ataluren at the beginning of the open label, extension trial, FEV_1 has remained stable since their transition to ataluren. We have not observed any increases in frequency of previously reported adverse events or significant numbers of new adverse events in this open label extension trial.

Change in %-predicted FEV_1 through week 96



Phase 2 clinical trials of ataluren for treatment of nmCF

In 2006, we completed two open label Phase 2 clinical trials of ataluren for the treatment of nmCF in adult patients. In these two trials, we enrolled a combined total of 47 patients age 18 years or older who were diagnosed with cystic fibrosis resulting from a nonsense mutation in the CFTR gene. We conducted the first trial at one site in Israel and the second trial at four sites in the United States. In 2008, we completed a third open label Phase 2 clinical trial of ataluren for the treatment of nmCF in pediatric and adolescent patients. We enrolled 30 patients between 6 and 18 years of age who were diagnosed with cystic fibrosis resulting from a nonsense mutation in the CFTR gene. We conducted this third trial at one site in France and two sites in Belgium. Each of these three trials had a treatment duration of 28 days. We also conducted an open label, extension trial with a treatment duration of three months for the patients who completed the 28-day trial in Israel. The goal of each of these trials was to obtain indications of pharmacological activity and to assess dose-response, safety and pharmacokinetics.

The trial designs for the three Phase 2 clinical trials with 28-day treatment durations were comparable and included two treatment cycles. Each cycle consisted of a two-week period of continuous ataluren treatment, and then a two-week follow-up period without ataluren treatment. During the two weeks of ataluren treatment one of the cycles, participants received ataluren at doses of 4 mg/kg in the morning, 4 mg/kg at midday and 8 mg/kg in the evening, for a total daily dose of 16 mg/kg. During the two weeks of ataluren treatment in the other cycle, the same participants received ataluren at doses of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg. We evaluated trial participants at the beginning and end of each two-week treatment period and follow-up period in each cycle. In the trial with a treatment duration of three months, patients received either ataluren at doses of 4 mg/kg in the morning, 4 mg/kg at midday and 8 mg/kg in the evening, for a total daily dose of 16 mg/kg, or ataluren at doses of 10 mg/kg in the morning, 10 mg/kg at midday and 20 mg/kg in the evening, for a total daily dose of 40 mg/kg.

The objective in each of these trials was to determine the change in CFTR-mediated chloride conductance in respiratory cells as measured between the beginning and end of treatment for each study participant. To make this determination, we measured the patient's TEPD. TEPD values are expressed in millivolts, or mV. A chloride conductance equal to or more electrically negative than -5.0 mV is generally considered to be in the normal range.

In the trials conducted in adults in Israel and in children and adolescents in France and Belgium, there were statistically significant improvements at the end of the ataluren treatment period in mean total chloride conductance and in the percentage of patients with a total chloride conductance response of at least a -5.0 mV improvement. There were also improvements in the percentage of patients with a chloride conductance in the normal range at the end of treatment. These results indicated the presence of pharmacological activity. These improvements were generally followed in the adult trials by reversions toward baseline with cessation of treatment during the follow-up period. In the trial conducted in the United States, we did not observe improvements in mean total chloride conductance.

Ataluren was generally well tolerated in these trials. Only one serious adverse event was considered possibly related to ataluren. Adverse events that were potentially drug-related were generally mild in severity. These adverse events included pain during urination in several patients. This issue resolved successfully with increased hydration. There were no clinically meaningful safety concerns identified in patients' physical examinations, vital sign measurements or electrocardiograms.

Phase 1 clinical trials of ataluren

We have completed two Phase 1 clinical trials of ataluren involving a total of 62 healthy volunteers. The first Phase 1 trial was a single-dose, safety and pharmacokinetic study with a placebo component, conducted in a total of 31 healthy volunteers between 18 and 30 years of age. In the first stage of the trial, subjects were enrolled at escalating dose levels ranging from 3 to 200 mg/kg. In this study, we determined that 100 mg/kg is the maximum tolerated dose based on the observation of increased frequency of headaches, dizziness and mild gastrointestinal events, such as nausea, vomiting and diarrhea, at the 150 mg/kg and 200 mg/kg doses. The drug was palatable, with no obvious odor or taste. In the second stage of this trial, we assessed the effect of food on the safety and pharmacokinetic profiles of ataluren at a dose of 50 mg/kg. This study provided us with pharmacokinetic data that indicated minimal alterations in the pharmacokinetic profile when ataluren was taken after a meal and supported giving ataluren with food to maintain plasma concentrations. The study also provided pharmacokinetic information allowing us to predict ataluren blood exposure levels in future studies.

The second Phase 1 trial was a multiple-dose, open label safety and pharmacokinetic study conducted in a total of 31 healthy volunteers between 18 and 30 years of age. In the first stage of the trial, subjects were enrolled at escalating twice-daily doses ranging from 10 to 50 mg/kg per dose taken with food for seven consecutive days. In the second stage of this trial, subjects were enrolled at a twice-daily dose of 50 mg/kg per dose for 14 days. In this study, there were no clinically significant adverse events reported at any dose tested, although we observed modest elevations of liver enzymes in some subjects. These elevated enzyme levels did not require cessation of ataluren administration, and enzyme levels typically normalized after completion of the treatment phase. As in the single-dose study, we were able to achieve and maintain plasma concentrations of ataluren that were predicted to have a therapeutic effect based on preclinical data. In the multiple-dose trial, as in the single-dose study, we sought to determine whether ataluren promoted improper read-through of normal stop codons. We assessed this by observing whether the trial participants produced improperly large forms of specified proteins. We did not observe any such improper protein formation.

Scientific background of post-transcriptional control processes

Post-transcriptional control processes are the events that occur in cells following the transcription of DNA to make mRNA. These processes regulate how long an mRNA molecule lasts in the cell and how efficiently the mRNA is used to produce its protein.

The majority of human protein-encoding genes are not contiguous but have an interrupted structure consisting of nucleotides that comprise the mRNA, called exons. The genetic information, encoded by exons, is interrupted by stretches of nucleotides called introns that are removed immediately after the gene is transcribed from DNA to the precursor messenger RNA, or pre-mRNA. The process of intron removal is called splicing.

The mRNA contains multiple regions that have specific functions. Although the protein coding region of mRNA contains the instructions to manufacture the protein, portions of mRNA that do not directly code for proteins, known as untranslated regions, or UTRs, are unique to specific mRNAs and are directly involved in the post-transcriptional control of protein production. Interactions of factors in the cell with the UTRs on the mRNA can modulate the translational efficiency of mRNA and how mRNA is degraded and eliminated from the cell.

Our approach

Our approach to drug discovery and development is to systematically target post-transcriptional control processes that can be modulated by small-molecule therapeutics. We believe that focusing on post-transcriptional control processes will enable us both to address known drug targets through new mechanisms of action and to pursue a broad range of targets that have previously not been amenable to drug discovery. We believe that a large number of promising post-transcriptional control drug targets remain unexploited, providing a significant opportunity for our integrated and systematic approach to drug discovery. This technology also has broad applicability to address intractable drug targets in a wide variety of diseases for which there is an unmet medical need, including genetic disorders, cancer, and musculoskeletal disorders, as well as inflammation, metabolic disorders, cardiovascular conditions and neurological disorders.

Our post-transcriptional control drug discovery technologies

We have developed and assembled an integrated set of proprietary technologies for the discovery of small molecules that target post-transcriptional control processes. Our technologies allow us to screen our compound library against targets in many different therapeutic areas in an expeditious and cost-effective manner. Our efforts span from target identification and characterization to the identification of selective lead molecules. From these lead molecules, our research team undertakes a chemical optimization program designed to select an appropriate development candidate. We refer to our technologies as GEMS, alternative splicing and nonsense suppression.

GEMS

We use our GEMS technology to identify molecules that modulate gene expression by targeting the post-transcriptional control processes that act through the UTRs of mRNA molecules. The UTRs of mRNA can have important roles in regulating protein production because they contain the instructions for determining the protein production efficiency and how long a given mRNA molecule will live within the cell.

We identify target proteins of potential biological and medical relevance to human disease and assess their regulation through UTRs and clinical feasibility. For targets that we select, we precisely identify the UTRs of the target gene.

We use proprietary assays to test our library of approximately 240,000 compounds to identify those that are likely to enhance or inhibit expression of the target gene by modulating the post-transcriptional control processes that act through that target's UTRs.

Alternative splicing

We use our alternative splicing technology to identify molecules that modulate mRNA splicing. Pre-mRNA splicing is a multi-step biochemical reaction. Approximately 94% of all human genes undergo splicing. In addition, through alternative splicing, one gene can often generate several mRNA products by including or excluding exons that can result in the mRNA being regulated differently or a different protein being produced. Altered regulation of alternative splicing is the direct cause of many human diseases, including many forms of cancer, Riley-Day syndrome (familial dysautonomia), myotonic dystrophy and spinal muscular atrophy.

We have developed a powerful high-throughput drug discovery technology that enables us to identify small molecule modifiers of pre-mRNA splicing. The technology relies on a sensitive quantification of mRNA directly in human cells or tissue samples. Using this technology, we have successfully identified orally bioavailable small molecules that correct splicing of the Survival Motor Neuron 2, or SMN2, gene, which is implicated in the genetic disorder spinal muscular atrophy. Based on this experience, we believe that other small molecule drug candidates can be rapidly identified that correct alternative splicing of genes, promote inclusion of specific exons into mRNA or force skipping of undesired exons from the mature mRNA. We believe that this technology is potentially widely applicable to a large number of target genes in all therapeutic areas.

Nonsense suppression

We use our nonsense suppression technology to identify molecules that promote or enhance nonsense suppression. The presence of a premature stop codon results in translation termination before a full-length protein can be produced. Our nonsense suppression technologies identify small molecules that increase nonsense suppression at the premature stop codon to produce a full-length protein. In addition to increasing read-through, small molecules that stabilize nonsense-containing mRNAs can enhance the effect of a compound that acts through the nonsense suppression mechanism.

Nonsense suppression also can be designed to identify molecules that can enhance the nonsense suppression effect of ataluren and other nonsense suppression agents to prevent the decay of nonsense-containing mRNAs. We have developed a high throughput screen to identify molecules that increase the level of nonsense-containing mRNAs. We can evaluate the effect of these molecules alone and in combination with ataluren in cell-based models of disease, identify lead compounds and initiate a chemical optimization program. We are currently in the process of evaluating compounds in preparation for an optimization program.

Preclinical development programs

SMN2 for spinal muscular atrophy

Using our alternative splicing technology, we have identified and are chemically optimizing several small molecule compounds, with the goal of selecting a lead development compound, for the treatment of spinal muscular atrophy. We have entered into a collaboration agreement with Roche and the SMA Foundation for the development and commercialization of these compounds. Roche is responsible for pursuing clinical development of compounds from the research program under the collaboration and then commercializing any resulting products. We also previously received $13.3 million in sponsored research funding for this program from the Spinal Muscular Atrophy Foundation.

Spinal muscular atrophy is a genetic neuromuscular disease characterized by muscle wasting and weakness. The disease generally manifests early in life. Spinal muscular atrophy is caused by defects in the Survival Motor Neuron 1, or SMN1, gene that encodes the survival motor neuron, or SMN, protein. The SMN protein is critical to the health and survival of the nerve cells in the spinal cord responsible for muscle contraction. A second gene, SMN2, is very similar to SMN1, except that SMN2 produces SMN protein that is less effective because, unlike SMN1, SMN2 does not include a particular nucleotide sequence known as exon 7. According to the SMA Foundation, spinal muscular atrophy is the leading genetic cause of death in infants and toddlers. The SMA Foundation estimates that spinal muscular atrophy affects approximately 10,000 to 25,000 children and adults in the United States and that between one in 6,000 and one in 10,000 children are born with the disease. There is currently no marketed therapy approved to treat the underlying cause of spinal muscular atrophy. Currently available treatments for spinal muscular atrophy are only palliative.

Using our alternative splicing technology, we have identified small molecule splicing modifiers that at very low concentrations in non-clinical studies involving cells from patients with spinal muscular atrophy increased both the inclusion of exon 7 in the SMN2 mRNA and the levels of SMN protein produced by SMN2. Importantly, in studies of mice with only the SMN2 gene, these compounds are orally bioavailable, penetrate the blood-brain barrier and increase full-length SMN mRNA and protein in various tissues. In these same mouse studies, treatment with these compounds resulted in increased survival, restoration of body weight, prevention of motor neuron loss and improved motor function. We expect to select a lead development compound in the first half of 2013.

Oncology—BMI1 program

We have selected a development candidate, PTC596, for the treatment of chemotherapy resistant cancers through the targeting of cancer stem cells. We are currently conducting IND-enabling preclinical studies with PTC596. We have received grant funding of $5.4 million for our BMI1 program from Wellcome Trust.

Cancer stem cells have been identified in numerous tumor types as a subpopulation of tumor cells that have the ability to initiate a tumor, produce other cancer cell types, move freely and proliferate throughout the body without attaching to other cells or surfaces and resist chemotherapy and radiotherapy. Many researchers believe that the resistance of cancer stem cells to chemotherapy and radiotherapy is a key factor in the failure of current cancer treatments. The BMI1 protein, which is overexpressed in many tumor subtypes, is a critical component of the polycomb repressive complex 1, or PRC1. PRC1 modulates gene expression that is important for cancer stem cell survival, maintenance, stabilization and differentiation. PRC1 is epigenetic, meaning that it is able to modify DNA directly to modulate gene expression without altering the nucleotide sequence in the genetic code. As a critical component of PRC1, the BMI1 protein regulates the self-renewal of adult blood and central nervous system stem cells that regulate cell growth.

PTC596 is an orally active small molecule that targets tumor stem cell populations by reducing the function, activity and amount of BMI1. PTC596 acts by altering and destroying the BMI1 protein through a process called phosphorylation. PTC596 has potently inhibited BMI1 function in multiple tumor cell lines. In *in vitro* tests, PTC596 has preferentially targeted chemotherapy resistant cancer stem cells. Specifically, PTC596 preferentially depleted cancer stem cells in assays with tumor cell lines from fibrosarcoma, prostate and colon cancers. Conversely, the cytotoxic chemotherapies carboplatin, temozolomide, methotrexate and indibulin enriched the population of cancer stem cells in this assay.

In animal cancer models using human tumors, weekly oral dosing of PTC596 provided tumor control, including reduction of tumor size. PTC596 and the commonly used chemotherapy paclitaxel were both

effective at controlling tumor growth in these animal models. However, PTC596, but not paclitaxel, decreased BMI1 levels, indicating a reduction in cancer stem cells. Consistent with this reduction in BMI1 levels, after transplanting tumor cells from one animal to another, the resulting tumors treated with PTC596 had lower levels of cancer stem cells than either untreated tumors or tumors treated with paclitaxel. PTC596 has been well tolerated at effective doses in animals. Preliminary data from these animal models suggest that PTC596 may preferentially target cancer stem cells without targeting normal stem cells.

Antibacterial Program

We have identified and are chemically optimizing several small molecule compounds for the treatment of life-threatening infections caused by multidrug-resistant Gram-negative bacteria. Our goal is to select lead development compounds that can be formulated for both intravenous and oral administration. Wellcome Trust awarded us a $5.0 million grant for this program, of which we have received approximately $2.5 million as of March 15, 2013.

The increasing prevalence of infections caused by multidrug-resistant bacteria is a global health problem and represents a critical unmet medical need. Many infections caused by multidrug-resistant pathogens occur in patients receiving medical care for serious conditions in hospitals, long-term acute care facilities, such as those providing wound care or ventilation, or nursing homes. Infections acquired in these settings, commonly referred to as nosocomial infections, frequently result in severe pneumonia and infections of the urinary tract and bloodstream. The majority of these cases of pneumonia and infections of the urinary tract and bloodstream are caused by Gram-negative bacteria.

We have identified a novel structural class of molecules that kill bacteria by targeting bacterial DNA synthesis. When tested using *in vitro* minimum inhibitory concentration, or MIC, assays, our compounds have demonstrated broad spectrum antibacterial activity against numerous Gram-negative bacteria, including *E. coli., A. baumannii, K. pneumoniae, H. influenzae, M. catarrhalis, N. gonorrhoeae, and Staphylococcus aureus.* We believe that the key differentiating factor of our compounds is their potent antibacterial activity against multidrug-resistant bacteria that are refractory to current drugs, including carbapenems and fluoroquinolones. Through chemical optimization, we have improved MIC levels 100-fold against Gram-negative pathogens and expanded the spectrum of activity to include select Gram-positive species, such as *Staphylococcus aureus*. We also have identified what we believe is the key structural feature that contributed to activity against drug-resistant pathogens. In animal studies, several analogs within this class of molecules have exhibited good exposure upon intravenous administration and protected mice against lethal *E. coli* infection.

Our collaborations and funding arrangements

We currently have ongoing collaborations with Roche and the SMA Foundation. We also have received grant funding from Wellcome Trust pursuant to funding agreements under which we have continuing obligations. In addition to these material collaboration and funding agreements, which are described in more detail below, we have an early stage collaboration and discovery agreement with AstraZeneca AB for the discovery and development of potential new therapies for cancer and other diseases.

Roche and the SMA Foundation

In November 2011, we entered into a license and collaboration agreement with Roche and the SMA Foundation pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our spinal muscular atrophy sponsored research program with the SMA Foundation, as described below, and to research, develop and commercialize other small molecule compounds with potential for therapeutic use in patients with spinal muscular atrophy. Pursuant to the license and collaboration agreement, Roche paid us an upfront non-refundable payment of $30.0 million. Roche has additional financial obligations described below.

Research and development. The agreement provides for a research and development collaboration under which we and Roche will conduct a program designed to further research and develop specified compounds from our pre-existing collaboration with the SMA Foundation, as described below, and to discover and develop new small molecule compounds that result in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA. During the research term, Roche has agreed to provide us with funding, based on an agreed-upon full-time equivalent rate, for an agreed-upon number of full-time equivalent employees that we contribute to the research program. The research term is for a minimum of two years from the effective date of the agreement and can be terminated by Roche any time thereafter upon 90 days' notice. Roche is responsible for pursuing worldwide clinical development of compounds from the research program.

Joint steering committee. The collaboration is governed by a joint steering committee consisting of an equal number of representatives of us, the SMA Foundation and Roche. We, the SMA Foundation and Roche have agreed that the members of the joint steering committee will act in good faith to cooperate with one another and seek agreement with respect to issues to be decided by the joint steering committee. In addition, we, the SMA Foundation and Roche have agreed to endeavor to make decisions by consensus, but if the joint steering committee cannot reach agreement after following a specified decision resolution procedure, Roche's decision will control. However, Roche may not exercise its final decision-making authority with respect to certain specified matters, including any decision that would increase our or the SMA Foundation's obligations, reduce our or the SMA Foundation's rights, expand Roche's rights, or reduce Roche's obligations under the license and collaboration agreement.

Commercialization. Roche is responsible for commercializing compounds and products from the collaboration. We have granted Roche worldwide exclusive licenses, with the right to grant sublicenses, to our patent rights and know-how with respect to such compounds and products.

We are eligible to receive up to an aggregate of $135 million in payments if specified development and regulatory milestones are achieved and up to an aggregate of $325 million in payments if specified sales milestones are achieved. We are also entitled to tiered single-digit to mid-teen royalties on worldwide net product sales of products developed pursuant to the collaboration. Roche's obligation to pay us royalties will expire generally on a country-by-country basis at the latest of the expiration of the last-to-expire patent covering a product in the given country, the expiration of regulatory exclusivity for that product in such country or 10 years from the first commercial sale of that product in such country. However, the royalties payable to us may be decreased in certain circumstances. For example, the royalty rate in a particular country is reduced if the product is not protected by patents in that country and no longer entitled to regulatory exclusivity in that country. We remain responsible for making any payments to the SMA Foundation that may become due under our pre-existing sponsored research agreement with the SMA Foundation.

Exclusivity. Roche has the exclusive right to develop and commercialize compounds from the collaboration. Furthermore, until November 2014, except in specified circumstances involving termination or certain acquisitions, neither we nor Roche is permitted, outside the collaboration, to use alternative splicing to identify any small molecule compound that results in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA or to engage in any research, development, manufacture or commercialization of any compound that such party knows or believes to be such a small molecule compound identified with alternative splicing.

Termination. Unless terminated earlier, the license and collaboration agreement will expire on the date when no royalty or other payment obligations are or will become due under the agreement.

Roche's termination rights under the license and collaboration agreement include the following:

- the right to terminate the agreement at any time after November 22, 2013 on a product-by-product and country-by-country basis upon three months' notice before the launch of the applicable product or upon nine months' notice thereafter; and

- the right to terminate the agreement in specified circumstances following a change of control of us.

The license and collaboration agreement provides that we or Roche may terminate the agreement in the event of an uncured breach by the other party of a material provision of the agreement, or in the event of the other party's bankruptcy or insolvency.

Upon termination of the collaboration agreement by Roche for convenience or termination by us as a result of Roche's breach, bankruptcy, change of control or patent challenge, we have the right to assume the development and commercialization of product candidates arising from the license and collaboration agreement. In that event, we may become obligated to pay royalties to Roche on sales of any such product.

SMA Foundation

In June 2006, we entered into a sponsored research agreement with the SMA Foundation under which we and the SMA Foundation have collaborated in the research and preclinical development of small molecule therapeutics for spinal muscular atrophy. Pursuant to the sponsored research agreement, as amended, the SMA Foundation provided us with $13.3 million in funding. The SMA Foundation is not obligated to provide any further funding under this agreement.

Research collaboration. The agreement established a research collaboration under which we identified and optimized compounds with the potential to treat spinal muscular atrophy by increasing production of the survival motor neuron, or SMN, protein. We expect to designate one of the compounds from the research program as a development candidate in the first half of 2013, and several other compounds from the research program have been designated as potential back-up compounds. As discussed above, we are also collaborating with the SMA Foundation and Roche to further develop these compounds.

Development and commercialization. We have agreed to use commercially reasonable efforts to develop and commercialize at least one product from compounds we advance from the research program, including performing specified activities within agreed timelines. As discussed above, we are also collaborating with the SMA Foundation and Roche to further develop these compounds.

Continuing financial obligations. We may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that we successfully develop and subsequently commercialize or, if we outlicense rights to a collaboration product, a specified percentage of certain payments we receive from our licensee. As discussed above, we have outlicensed rights to Roche pursuant to a license and collaboration agreement. We are not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Our obligation to make such payments would end upon our payment to the SMA Foundation of a specified amount, which we refer to as the repayment amount.

Reversion rights. In specified circumstances, including those involving our decision to discontinue development or commercialization of a collaboration product, our uncured failure to meet agreed timelines or those that might arise following our change of control, we may be obligated to grant the SMA Foundation exclusive or non-exclusive sublicensable rights under our intellectual property, in certain collaboration products, among other rights, to assume the development and commercialization of such

collaboration products and to provide the SMA Foundation with other transitional assistance, which we refer to as a reversion. In some such cases, we may be entitled to receive licensing fee payments from the SMA Foundation and single-digit royalties on sales of the applicable collaboration product, which amounts we collectively refer to as reversion payments. In other cases, the SMA Foundation is not required to make any payments to us in connection with the licenses it receives from us.

Termination. Unless terminated earlier, the sponsored research agreement will continue until the earliest of the SMA Foundation's receipt of the repayment amount or, if there was a reversion, either our receipt of all reversion payments that the SMA Foundation may be obligated to make to us or, if the SMA Foundation is not obligated to make reversion payments, the expiration of the last-to-expire patent we licensed to the SMA Foundation in connection with such reversion. The sponsored research agreement provides that either party may terminate the agreement in the event of an uncured material breach by the other party or in the event of the other party's bankruptcy or insolvency.

Wellcome Trust (BMI1 for oncology)

In May 2010, we entered into a funding agreement with Wellcome Trust for the research and development of small molecule compounds that selectively decrease the production of BMI1 expression in tumor stem cells. Pursuant to the funding agreement, Wellcome Trust awarded us a $5.4 million grant, of which approximately $0.9 million was paid in connection with execution of the agreement and the balance of which was paid based on our achievement of specified milestones.

Research program. We have agreed to use reasonable efforts to achieve each specified research program milestone on or before its corresponding agreed target date. We have designated PTC596 as an experimental drug candidate. The research program term began on the effective date and ends on the earlier of completion of the research program or three years after the effective date.

Development and commercialization. We own all intellectual property that arises from the conduct of the research program, which we refer to as program intellectual property, and are responsible for developing and commercializing the program intellectual property, including PTC596 and other compounds. However, we will require Wellcome Trust's written consent prior to any such development or commercialization. If Wellcome Trust withholds such consent and we and Wellcome Trust are not able to resolve Wellcome Trust's concerns, the parties have agreed to follow a specified dispute resolution procedure that gives neither party final decision-making authority.

Continuing financial obligations. To the extent that we develop and commercialize program intellectual property on a for-profit basis ourselves, we may become obligated to pay to Wellcome Trust development and regulatory milestone payments of up to an aggregate of $35.6 million and single-digit royalties on sales of any research program product. Our obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. To the extent that we develop and commercialize program intellectual property on a for-profit basis through outlicensing, we will be obligated to pay to Wellcome Trust a specified share of any consideration we receive from our licensee. We would incur no payment obligations to Wellcome Trust to the extent that we elect to develop and commercialize program intellectual property on a non-profit basis.

Reversion rights. If we fail to take reasonable steps to develop or commercialize program intellectual property during specified timeframes, we may be obligated to grant exclusive rights to Wellcome Trust or its nominee under the program intellectual property, along with non-exclusive rights under our background intellectual property, so that Wellcome Trust or its nominee can assume such development and

commercialization. If we grant such a license, we would be entitled to a share of any consideration received by Wellcome Trust in connection with any subsequent development or commercialization of program intellectual property on a for-profit basis, which share would be proportionate to our contribution to the development and commercialization.

Termination. Unless terminated earlier, the funding agreement will continue until the research program has ended, the last-to-expire of the patents in the program intellectual property has expired, any agreement entered into for the exploitation of the program intellectual property or our background intellectual property has expired, and there are no remaining payment obligations relating to the exploitation of the program intellectual property or certain of our other intellectual property.

Wellcome Trust's rights under the funding agreement include the right to terminate the agreement under specified circumstances, including if:

- according to a team of experts from Wellcome Trust, an uncorrected serious failure exists in the progress, management or conduct of the research program, or an uncorrected major external scientific, technical or commercial barrier exists that means the research program is unlikely to succeed in its objectives;
- we cease or threaten to cease to carry on our business or operations necessary for the completion of our obligations under the funding agreement;
- in Wellcome Trust's reasonable opinion, an act or omission by us is incompatible with or has an adverse effect on Wellcome Trust's charitable objectives or reputation or on our ability to comply with our obligations under the funding agreement;
- we enter into any transaction involving the program intellectual property or our background intellectual property without Wellcome Trust's prior written consent;
- specified events take place relating to our principal investigator for the research program; or
- specified situations exist following a change of control of us.

The funding agreement provides that either party may terminate the agreement in the event of an uncured material breach by the other party or in the event of the other party's bankruptcy or insolvency.

Except as noted below, certain specified rights and obligations of the parties will generally survive termination of the funding agreement, including Wellcome Trust's right to receive payments from us with respect to development and commercialization of program intellectual property on a for-profit basis.

If the funding agreement terminates prior to the end of the research program, we are obligated to return all funding we received from Wellcome Trust that is unspent at the date of termination, after deduction of costs and non-cancellable commitments incurred prior to such date.

If Wellcome Trust terminates the funding agreement in specified circumstances, including as a result of our material breach, bankruptcy or insolvency, or following our change of control, we may be obligated to assign to Wellcome Trust ownership of the program intellectual property, grant to Wellcome Trust royalty-free non-exclusive rights under our background intellectual property for the continuation of the research program, if applicable, and the development and commercialization of program intellectual property, and provide Wellcome Trust with other specified transitional assistance.

If we terminate the funding agreement in specified circumstances, including as a result of Wellcome Trust's uncured material breach or bankruptcy or insolvency, Wellcome Trust's right to receive payments from us with respect to development and commercialization of program intellectual property on a for-profit basis, as well as certain other specified rights, will terminate.

Wellcome Trust (antibacterial)

In December 2011, we entered into an additional funding agreement with Wellcome Trust for the research and development of small molecule compounds that target life-threatening infections caused by multidrug-resistant Gram-negative bacteria. Pursuant to the funding agreement, Wellcome Trust awarded us a $5.0 million grant, of which approximately $1.7 million was paid in connection with execution of the agreement. The balance of the grant is payable based on our achievement of three specified milestones. As of March 15, 2013, we have achieved one of these milestones, triggering additional payments to us of $1.6 million.

Research program. We have agreed to use reasonable efforts to achieve each specified research program milestone on or before its corresponding agreed target date. The research program term began on the effective date of the agreement and ends on the earlier of completion of the research program or three years after the effective date.

Development and commercialization. We own all intellectual property that arises from the conduct of the research program, which we refer to as program intellectual property, and have the first right to develop and commercialize the program intellectual property, including compounds, provided that we obtain Wellcome Trust's written consent prior to any such development or commercialization. If Wellcome Trust withholds such consent and we and Wellcome Trust are not able to resolve Wellcome Trust's concerns, the parties have agreed to follow a specified dispute resolution procedure that gives neither party final decision-making authority.

Continuing financial obligations. To the extent that we develop and commercialize program intellectual property on a for-profit basis ourselves, we may become obligated to pay to Wellcome Trust development and regulatory milestone payments of up to an aggregate of $33.3 million and single-digit royalties on sales of any research program product. Our obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. To the extent that we develop and commercialize program intellectual property on a for-profit basis through outlicensing, we will be obligated to pay to Wellcome Trust a specified share of any consideration we receive from our licensee. We would incur no payment obligations to Wellcome Trust to the extent that we elect to develop and commercialize program intellectual property on a non-profit basis.

Reversion rights. If we fail to take reasonable steps to develop or commercialize program intellectual property during specified timeframes, we may be obligated to grant exclusive rights to Wellcome Trust or its nominee under the program intellectual property, along with non-exclusive rights under our background

intellectual property, so that Wellcome Trust or its nominee can assume such development and commercialization. If we grant such a license, we would be entitled to a share of any consideration received by Wellcome Trust in connection with any subsequent development or commercialization of program intellectual property on a for-profit basis, which share would be proportionate to our contribution to the development and commercialization.

Termination. Unless terminated earlier, the funding agreement will continue until we have received the full amount of the grant, the research program has ended, the last-to-expire of the patents in the program intellectual property has expired, any agreement entered into for the exploitation of the program intellectual property or our background intellectual property has expired, and there are no remaining payment obligations relating to the exploitation of the program intellectual property or our background intellectual property.

Wellcome Trust's termination rights under the funding agreement include the right to terminate the funding agreement under specified circumstances, including if:

- according to a team of experts from Wellcome Trust, an uncorrected serious failure exists in the progress, management or conduct of the research program, or an uncorrected major external scientific, technical or commercial barrier exists that means the research program is unlikely to succeed in its objectives;
- we cease or threaten to cease to carry on our business or operations necessary for the completion of our obligations under the funding agreement;
- in Wellcome Trust's reasonable opinion, an act or omission by us is incompatible with or has an adverse effect on Wellcome Trust's charitable objectives or reputation or on our ability to comply with our obligations under the funding agreement;
- we enter into any transaction involving the program intellectual property or our background intellectual property without Wellcome Trust's prior written consent;
- specified events take place relating to our principal investigator for the research program; or
- specified situations exist following a change of control of us.

The funding agreement provides that either party may terminate the agreement in the event of an uncured material breach by the other party or in the event of the other party's bankruptcy or insolvency.

Except as noted below, certain specified rights and obligations of the parties will generally survive termination of the funding agreement, including Wellcome Trust's right to receive payments from us with respect to development and commercialization of program intellectual property on a for-profit basis.

If the funding agreement terminates prior to the end of the research program, we are obligated to return all funding we received from Wellcome Trust that is unspent at the date of termination (after deduction of costs and non-cancellable commitments incurred prior to such date).

If Wellcome Trust terminates the funding agreement in specified circumstances, including as a result of our material breach, bankruptcy or insolvency, or following our change of control, we may be obligated to assign to Wellcome Trust ownership of the program intellectual property, grant to Wellcome Trust royalty-free non-exclusive rights under our background intellectual property for the continuation of the research program (if applicable) and the development and commercialization of program intellectual property, and provide Wellcome Trust with other specified transitional assistance.

If we terminate the funding agreement in specified circumstances, including as a result of Wellcome Trust's uncured material breach or bankruptcy or insolvency, Wellcome Trust's right to receive payments from us with respect to development and commercialization of program intellectual property on a for-profit basis, as well as certain other specified rights, will terminate.

Intellectual property

Patents and trade secrets

Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates, technology and know-how, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing U.S. and certain foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development of our business, where patent protection is available. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

As of January 31, 2013, we owned or exclusively licensed a total of 55 U.S. patents and 73 U.S. patent applications, including original filings, continuations and divisional applications, as well as numerous foreign counterparts to many of these patents and patent applications. Our patent portfolio includes patents and patent applications with claims directed to the composition of matter, pharmaceutical formulation and methods of use of many of our compounds, including ataluren.

The patent rights relating to ataluren owned by us consist of thirteen issued U.S. patents relating to composition of matter, methods of use, formulation and methods of manufacture and multiple pending patent applications relating to composition of matter, methods of use, formulation, dosing and methods of manufacture. The issued U.S. patents relating to composition of matter are currently scheduled to expire in 2024, and all U.S. patents that issue from U.S. patent applications relating to composition of matter would also be scheduled to expire in 2024. An issued U.S. patent relating to therapeutic method of use is currently scheduled to expire in 2027. All of these patent rights are also the subject of pending counterpart patent applications in a number of other jurisdictions, including Europe and Japan. We own two issued European patents relating to dosing and methods of manufacture of ataluren, and multiple pending European patent applications relating to composition of matter, methods of use, formulation, dosing and methods of manufacture of ataluren. The issued European patents are currently scheduled to expire in 2026 and 2027, and any European patent that issues from the pending European patent application relating to composition of matter would currently be expected to expire in 2024. The anticipated expiration dates referred to above are without regard to potential patent term extension, patent term adjustment or other market exclusivity that may be available to us.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a U.S. patent that covers a drug, biological product or medical device approved pursuant to a pre-market approval, or PMA, may also be eligible for patent term extension when FDA approval is granted, provided statutory and regulatory requirements are met. The length of the patent term extension is related to the length of time the drug is under regulatory review while the patent is in force. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration date

set for the patent. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be granted an extension and only those claims reading on the approved drug may be extended. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our product candidates receive approval by the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

License agreements

We are a party to a number of license agreements under which we license patents, patent applications and other intellectual property from third parties. We enter into these agreements to augment our proprietary intellectual property portfolio. The licensed intellectual property covers some of the compounds that we are researching and developing, some post-transcriptional control targets and some of the scientific processes that we use. These licenses impose various diligence and financial payment obligations on us. We expect to continue to enter into these types of license agreements in the future.

Manufacturing

We do not currently own or operate manufacturing facilities for the production of clinical or commercial quantities of ataluren or for the compounds that we are testing in our preclinical programs. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates and any products that we may develop, other than small amounts of compounds that we synthesize ourselves for preclinical testing.

We obtain our supply of the bulk drug substance for ataluren from a single third-party manufacturer. We engage a separate manufacturer to provide fill and finish services for the finished product that we are using in our ongoing clinical trials of ataluren. We are in the process of qualifying an additional manufacturer for the supply of bulk drug substance and for fill and finish services for our future clinical trials of ataluren. We obtain our supplies of the product candidates from these manufacturers pursuant to agreements that include specific supply timelines and volume expectations. If any of these manufacturers should become unavailable to us for any reason, we believe that there are a number of potential replacements, although we might incur some delay in identifying and qualifying such replacements.

All of our drug candidates are organic compounds of low molecular weight, generally called small molecules. We have selected these compounds not only on the basis of their potential efficacy and safety, but also for their ease of synthesis and reasonable cost of their starting materials. In particular, ataluren is

manufactured in reliable and reproducible synthetic processes from readily available starting materials. The chemistry is amenable to scale up and does not require unusual equipment in the manufacturing process. We expect to continue to develop drug candidates that can be produced cost-effectively at contract manufacturing facilities.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or are less expensive than any products that we may develop. In addition, our ability to compete may be affected because in some cases insurers or other third-party payors seek to encourage the use of generic products. This may have the effect of making branded products less attractive, from a cost perspective, to buyers.

The competition for ataluren includes the following:

- ***Ataluren for nmDMD.*** There are currently no marketed therapeutics approved to treat the underlying cause of nmDMD. Current treatments seek to address symptoms through supportive care measures, such as bracing, joint stretching exercises, tendon release surgery, wheelchair use and assisted ventilation. Corticosteroids, such as prednisone and deflazacort are often prescribed to treat some of the symptoms of the disease. We believe that ataluren is the only product candidate in clinical trials that is designed to treat the underlying cause of nmDMD by restoring dystrophin activity. Other biopharmaceutical companies are developing treatments for Duchenne muscular dystrophy based on a different scientific approach known as exon-skipping. Prosensa Therapeutics is developing a product candidate, PRO051, based on exon-skipping that is currently in Phase 3 clinical development in collaboration with GlaxoSmithKline. Sarepta Therapeutics is developing a product candidate, Eteplirsen, based on exon-skipping that is currently in Phase 2b clinical development. We do not believe that either PRO051 or Eteplirsen is applicable for the treatment of patients with nmDMD. Summit Corporation also has a product candidate in early clinical development designed to increase the production of the protein utrophin, which is functionally similar to dystrophin, to treat Duchenne muscular dystrophy. In addition, Pfizer has a potentially muscle-enhancing product candidate in Phase 2 clinical development for muscular dystrophy.

- ***Ataluren for nmCF.*** There are currently no marketed therapeutics approved to treat the underlying cause of nmCF. In 2012, the FDA approved Kalydeco (ivacaftor), a CFTR potentiator developed by Vertex

Pharmaceuticals, as a treatment for cystic fibrosis in patients six years of age and older who have a type of mutation in the CFTR gene known as a gating mutation. Other current treatments for cystic fibrosis are designed to alleviate the symptoms of the disease and depend upon the stage of the disease and the organs involved. Clearing mucus from the lungs is an important part of the daily cystic fibrosis treatment regimen. Chest physical therapy is a form of airway clearance that involves vigorous clapping on the back and chest to dislodge the thick mucus from the lungs. Other treatments for cystic fibrosis include TOBI (tobramycin), an aerosolized antibiotic used to treat lung infections that is marketed by Chiron Corporation, and Pulmozyme, a mucus-thinning drug shown to reduce the number of lung infections and improve lung function, that is marketed by Genentech, Inc. We believe that ataluren is the only product candidate in clinical trials that is designed to treat the underlying cause of nmCF by restoring CFTR activity. Vertex Pharmaceuticals also is developing two other product candidates for the treatment of cystic fibrosis in patients who have a type of mutation in the CFTR gene known as a processing block mutation, one of which is in Phase 2 clinical development in combination with Kalydeco. We do not believe that Kalydeco or these two product candidates under development by Vertex Pharmaceuticals are applicable for the treatment of patients with nmCF.

The key competitive factors affecting the success of ataluren are likely to be its efficacy, safety, convenience, price and the availability of coverage and reimbursement from government and other third-party payors.

Sales and marketing

If we receive regulatory approval for our product candidates, we plan to commence commercialization activities by building a focused sales and marketing organization complemented by selective distribution, co-promotion and other arrangements with leading pharmaceutical or biotechnology collaborators.

We generally expect to retain commercial rights for our product candidates for which we receive marketing approvals in situations in which we believe it is possible to access the market through focused, specialized sales force. In particular, we believe that such a sales force could address the community of pulmonologists and neurologists who are the key specialists in treating cystic fibrosis and Duchenne muscular dystrophy, for which we are developing ataluren. Accordingly, if ataluren is approved, we plan to initially build our own internal sales teams to target these specialists.

We also plan to build key capabilities, such as marketing, market access, sales management and medical affairs, to implement marketing and medical strategies for any products that we market through our own sales organization and to oversee and support our sales force. The responsibilities of the marketing organization would include developing educational initiatives with respect to approved products and establishing relationships with thought leaders in relevant fields of medicine.

Government regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, approval, manufacturing, labeling, post-approval monitoring and reporting, packaging, promotion, storage, advertising, distribution, marketing and export and import of pharmaceutical products such as those we are developing. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

U.S. government regulation

In the United States, the information that must be submitted to the FDA in order to obtain approval to market a new drug varies depending upon whether the drug is a new product whose safety and efficacy have not previously been demonstrated in humans or a drug whose active ingredients and certain other properties are the same as those of a previously approved drug. A New Drug Application, or NDA, is the vehicle through which the FDA approves a new pharmaceutical product for sale and marketing in the United States.

The NDA approval process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or the FDCA, and implementing regulations. Failures to comply with the applicable FDA requirements at any time during the product development process or approval process may result in a delay of approval or administrative or judicial sanctions. These sanctions could include the FDA's imposition of a clinical hold on trials, refusal to approve pending applications, withdrawal of an approval, issuance of warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

To market a new drug, a sponsor generally must undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies under the FDA's good laboratory practices regulations;
- submission to the FDA of an investigational new drug application, or IND, for clinical testing, which must become effective before clinical trials may begin and which must include independent Institutional Review Board, or IRB, approval before the trials may be initiated;
- performance of adequate and well-controlled clinical trials in accordance with Good Clinical Practices to establish the safety and efficacy of the product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA Advisory Committee meeting, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practices, or cGMP, which require that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity; and
- FDA review and approval of the NDA.

Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. An IND applicant must submit the results of the preclinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some preclinical testing may continue after the IND is submitted. The IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about issues such as the conduct of the trials as outlined in the IND. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed. In other words, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB must approve the protocol and amendments. All research subjects or their legally authorized representatives must provide their informed consent in writing.

Clinical trials typically are conducted in three sequential phases, but the phases may overlap or be combined. Phase 1 trials usually involve the initial introduction of the investigational drug into human subjects. Phase 1 trials may be conducted in patients or healthy volunteers to evaluate the product's safety, dosage tolerance and pharmacokinetics and, if possible, seek to gain an early indication of its effectiveness.

Phase 2 trials usually involve controlled trials in a larger but still relatively small number of subjects from the relevant patient population to:

- evaluate dosage tolerance and appropriate dosage;
- identify possible short-term adverse effects and safety risks; and
- provide a preliminary evaluation of the efficacy of the drug for specific indications.

Phase 2 trials are sometimes denoted by companies as Phase 2a or Phase 2b trials. Phase 2a trials typically represent the first human clinical trial of a drug candidate in a smaller patient population and are designed to provide earlier information on drug safety and efficacy. Phase 2b trials typically involve larger numbers of patients or longer durations of therapy and may involve comparison with placebo, standard treatments or other active comparators.

Phase 3 trials usually further evaluate clinical efficacy and test further for safety in an expanded patient population. Phase 3 trials usually involve comparison with placebo, standard treatments or other active comparators. These trials are intended to establish the overall risk-benefit profile of the product and provide an adequate basis for physician labeling. Phase 3 trials are usually larger, more time consuming, more complex and more costly than Phase 1 and Phase 2 trials.

Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within any specified period, if at all. Furthermore, the FDA or we may suspend or terminate clinical trials at any time on various grounds, including a finding that the subjects are or would be exposed to an unreasonable and significant risk of illness or injury. Similarly, an IRB can suspend or terminate approval of a clinical trial if the trial is not being conducted in accordance with the IRB's requirements or if the research has been associated with unexpected serious harm to patients. The FDA typically requires that an NDA include data from two adequate and well-controlled clinical trials, but approval may be based upon a single adequate and well-controlled clinical trial plus confirmatory evidence. In some cases, the FDA may condition approval of an NDA on the applicant's agreement to conduct additional clinical trials to further assess the drug's safety and effectiveness after NDA approval. Such post-approval trials are typically referred to as Phase 4 studies.

The FDA's accelerated approval process allows for potentially faster development and approval of certain drugs intended to treat serious or life-threatening illnesses that provide meaningful therapeutic benefit to patients over existing treatments. Under the accelerated approval process, the adequate and well-controlled clinical trials conducted with the drug establish that the drug has an effect on a "surrogate" endpoint that is reasonably likely to predict clinical benefit or on the basis of an effect on a

clinical benefit other than survival or irreversible morbidity. Drugs approved through the accelerated approval process are subject to certain post-approval requirements, including that the applicant complete Phase 4 clinical trials to demonstrate the drug's clinical benefit. If the trials fail to verify the clinical benefit of the drug, the FDA may withdraw approval of the application through a streamlined process.

Assuming successful completion of the required clinical testing, the results of the preclinical studies and of the clinical trials, together with other detailed information, including proposed labeling and information on the chemistry, manufacture and composition of the product, are submitted to the FDA in the form of an NDA requesting approval to market the product for one or more indications. In most cases, the NDA must be accompanied by a substantial user fee. The FDA will initially review the NDA for completeness before it accepts the NDA for filing. After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether a product is safe and effective for its intended use and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity.

Under the Pediatric Research Equity Act of 2003, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted. PREA compliance may be required if approval is sought for other indications for which the drug has not received orphan designation.

The FDA will typically inspect one or more clinical sites to assure compliance with GCP before approving an NDA. The FDA also will inspect the facility or the facilities at which the product is manufactured before the NDA is approved. The FDA will not approve the product unless cGMP compliance is satisfactory. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The testing and approval process requires substantial time, effort and financial resources, and each may take years to complete. Data obtained from clinical trials are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all.

We may encounter difficulties or unanticipated costs in our efforts to secure necessary FDA approvals, which could delay or preclude us from marketing our products. The FDA may refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. The advisory committee process may cause delays in the approval timeline. The FDA is not bound by the recommendation of an advisory committee, but it considers such recommendations carefully, particularly any negative recommendations or limitations, when making drug approval decisions.

The FDA may limit the indications for use, approve narrow labeling relegating a drug to second-line or later-line use, add limitations of use to the labeling or place other conditions on approvals, which could restrict the marketing of the products. After approval, some types of changes to the approved product,

such as adding new indications, which may itself require further clinical testing, or changing the manufacturing process are subject to further FDA review and approval.

Post-approval requirements

After FDA approval of a product is obtained, we are required to comply with a number of post-approval requirements. Holders of an approved NDA must report certain adverse reactions and production problems to the FDA, provide updated safety and efficacy information, and comply with requirements concerning advertising and promotional labeling for their products. As a condition of approval of an NDA, the FDA may require post marketing testing and surveillance to monitor the product's safety or efficacy.

The FDA also has the authority to require a drug-specific risk evaluation and mitigation strategy, or REMS, to ensure the safe use of the drug. In determining whether a REMS is necessary, the FDA must consider the size of the population likely to use the drug, the seriousness of the disease or condition to be treated, the expected benefit of the drug, the duration of treatment, the seriousness of known or potential adverse events, and whether the drug is a new molecular entity. A REMS may be required to include various elements, such as a medication guide or patient package insert, a communication plan to educate health care providers of the drug's risks, limitations on who may prescribe or dispense the drug, or other measures that the FDA deems necessary to assure the safe use of the drug. In addition, the REMS must include a timetable to assess the strategy at 18 months, three years, and seven years after the strategy's approval. The FDA may also impose a REMS requirement on an approved drug if the FDA determines, based on new safety information, that a REMS is necessary to ensure that the drug's benefits outweigh its risks.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Although physicians may prescribe a drug for off-label uses, manufacturers may only promote for the approved indications and in accordance with the approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, that regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Also, quality control and manufacturing procedures must continue to conform to cGMP after approval. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which imposes certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Future FDA inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved NDA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing.

Newly discovered or developed safety or effectiveness data or other information may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established that could delay or prevent FDA approval of our products under development or negatively impact the marketing of any future approved products.

Orphan drug designation

We have received orphan drug designation from the FDA for ataluren for the treatment of nmCF and nmDMD. The FDA may grant orphan drug designation to drugs intended to treat a "rare disease or condition," which is defined as a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Orphan drug designation can provide opportunities for grant funding towards clinical trial costs, tax advantages and FDA user-fee benefits. In addition, if a product which has an orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors may receive approval of different drugs or biologics for the indications for which the orphan product has exclusivity.

Fast track designation

We have obtained fast track designation from the FDA for our product candidate ataluren for the treatment of nmCF and nmDMD. The FDA's fast track program is a process designed to facilitate the development and review of new drugs that are intended to treat serious or life threatening conditions and that demonstrate the potential to address unmet medical needs for the conditions. Fast track designation applies to the product for the specific indication for which it is being studied. Thus, it is the development program for a specific drug for a specific indication that receives fast track designation. The sponsor of a product designated as being in a fast track drug development program may engage in close early communication with the FDA including through timely meetings and feedback on clinical trials. Products in the fast track drug development program also may receive priority review or accelerated approval, and sponsors may be able to submit portions of an application on a rolling basis rather than as one complete submission. The FDA may notify a sponsor that its program is no longer classified as a fast track development program if the fast track designation is no longer supported by emerging data or the designated drug development program is no longer being pursued.

Hatch-Waxman exclusivity

Market and data exclusivity provisions under the FDCA can delay the submission or the approval of certain applications for competing products. The FDCA provides a five-year period of non-patent data exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a

505(b)(2) NDA submitted by another company that references the previously approved drug. However, an ANDA or 505(b)(2) NDA may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of market exclusivity for an NDA, 505(b)(2) NDA, or supplement to an existing NDA or 505(b)(2) NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant, are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages, strengths or dosage forms of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent market exclusivity in the United States and, if granted, provides for the attachment of an additional six months of market protection to the term of any existing regulatory exclusivity, including the non-patent exclusivity periods described above. This six-month exclusivity may be granted based on the voluntary completion of a pediatric study or studies in accordance with an FDA-issued "Written Request" for such a study or studies.

Regulation outside the United States

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

Regulation in the European Union

We have obtained an orphan medicinal product designation from the European Commission, following an evaluation by the EMA's Committee for Orphan Medicinal Products, for ataluren for the treatment of nmDMD and nmCF. The European Commission can grant orphan medicinal product designation to products for which the sponsor can establish that it is intended for the diagnosis, prevention, or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the European Union, or (2) a life threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that sales of the drug in the European Union would generate a sufficient return to justify the necessary investment. In addition, the sponsor must establish that there is no other satisfactory method approved in the European Union of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation is not a marketing authorization. It is a designation that provides a number of benefits, including fee reductions, regulatory assistance, and the possibility to apply for a centralized E.U. marketing authorization, as well as 10 years of market exclusivity following a marketing authorization. During this market exclusivity period, neither the European Medicines Agency, nor

the European Commission nor the Member States can accept an application or grant a marketing authorization for a 'similar medicinal product.' A 'similar medicinal product' is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may be reduced to six years if, at the end of the fifth year, it is established that the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. In addition, a competing similar medicinal product may in limited circumstances be authorized prior to the expiration of the market exclusivity period, including if it is shown to be safer, more effective or otherwise clinically superior to our product. Our product can lose orphan designation, and the related benefits, prior to us obtaining a marketing authorization if it is demonstrated that the orphan designation criteria are no longer met.

To obtain regulatory approval of a drug under the European Union's regulatory systems and authorization procedures, an applicant may submit MAAs under a centralized, decentralized, or national procedure. The centralized procedure is compulsory for certain medicinal products, including orphan medicinal products, like ataluren for the treatment of nmDMD and nmCF, and medicinal products produced by certain biotechnological processes, and optional for certain other innovative products. The centralized procedure enables applicants to obtain a marketing authorization that is valid in all E.U. member states based on a single application. Under the centralized procedure, the EMA's Committee for Human Medicinal Products, or CHMP, is required to adopt an opinion on a valid application within 210 days, excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions. An accelerated assessment can be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days. After the adoption of the CHMP opinion, a decision on the MAA must be adopted by the European Commission, after consulting the European Union member states, which in total can take more than 60 days.

The decentralized procedure allows applicants to file identical applications to several E.U. member states and receive simultaneous national approvals based on the recognition by E.U. member states of an assessment by a reference member state. The national procedure is only available for products intended to be authorized in a single E.U. member state. A mutual recognition procedure similar to the decentralized procedure is available when a marketing authorization has already been obtained in at least one European Union member state.

In specific circumstances, E.U. legislation enables applicants to obtain a conditional marketing authorization prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for products designated as orphan medicinal products, if (1) the risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) the product fulfills unmet medical needs, and (4) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations.

Prior to obtaining a marketing authorization in the European Union, applicants have to demonstrate compliance with all measures included in an EMA-approved Paediatric Investigation Plan, or PIP, covering

all subsets of the paediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver, or (3) a deferral for one or more of the measures included in the PIP. In the case of orphan medicinal products, completion of an approved PIP can result in an extension of the aforementioned market exclusivity period from ten to twelve years.

In the European Union, independently generated data submitted as part of a full marketing authorization application dossier are protected by regulatory data protection ('data exclusivity') for a period of eight years from the granting of a marketing authorization for a 'reference product'. This means that for a period of eight years, competent authorities may not accept marketing authorization applications that rely on the independently generated data in the marketing authorization dossier of the reference product. Generic medicinal products that rely on the independently generated data of the reference product may not be placed on the market for 10 years from the granting of the initial marketing authorization for the reference medicinal product. These periods of data exclusivity and market exclusivity do not prevent other companies from obtaining a marketing authorization based on their own independently generated data.

If we obtain authorization for a medicinal product in the European Union, we will be required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. We will, for example, have to comply with the E.U.'s stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. Other requirements relate to, for example, the manufacturing of products and active pharmaceutical ingredients in accordance with good manufacturing practice standards. E.U. regulators may conduct inspections to verify our compliance with applicable requirements, and we will have to continue to expend time, money and effort to remain compliant. Non-compliance with E.U. requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties in the E.U. Similarly, failure to comply with the E.U.'s requirements regarding the protection of individual personal data can also lead to significant penalties and sanctions. Individual E.U. member states may also impose various sanctions and penalties in case we do not comply with locally applicable requirements.

Pharmaceutical pricing and reimbursement

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of pharmaceuticals have been a focus of this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 expanded Medicare coverage for drug purchases by the elderly and changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this law may decrease the coverage and reimbursement rate that we may receive for any approved products. Likewise, healthcare reform measures under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, referred to together as the Affordable Care Act, contain provisions that may reduce the profitability of drug products by increasing rebates for covered outpatient drugs sold to Medicaid programs, extending the Medicaid rebate to Medicaid managed care plans, mandating discounts for certain Medicare Part D beneficiaries, and imposing annual fees based on pharmaceutical companies' share of sales to federal healthcare programs, among other provisions.

In this healthcare regulatory climate, there may be significant delays in and impediments to obtaining coverage and reimbursement for newly approved drugs. Coverage by federal healthcare programs may be

more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities' coverage of the same products. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the extent to which the costs of the products will be covered and reimbursed by third-party payors, including government healthcare programs such as Medicare and Medicaid, private health insurers and other organizations. Obtaining reimbursement for orphan drugs may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement in the use of a higher priced drug.

The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA approved products for a particular indication. Third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. Our product candidates may not be considered cost-effective. In the future, we may need to conduct direct head-to-head studies to demonstrate clinical superiority and cost-effectiveness. Our product candidates may not be considered clinically superior and cost-effective to competitor products.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and other third-party payors fail to provide adequate coverage and reimbursement. In addition, there is an increasing emphasis on managed care in the United States that may negatively impact pharmaceutical pricing.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerably pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various E.U. member states, and parallel distribution (arbitrage between low-priced and high-priced member states), can further reduce prices. In some countries we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidate to other available therapies in order to obtain reimbursement or pricing approval.

U.S. fraud and abuse laws

Any present or future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may restrict certain marketing and contracting practices. These laws include anti-kickback and false claims statutes.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, in cash or kind, to induce or reward either the referral of an individual for, or the purchase, or order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. This statute has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others.

The federal False Claims Act imposes civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. The government and *qui tam* relators have brought False Claims Act actions against pharmaceutical companies on the theory that their practices have caused false claims to be presented to the government. There is also a separate false claims provision imposing criminal penalties.

The Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

HIPAA also imposes criminal liability for knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services.

The federal Physician Sunshine Act requirements under the Affordable Care Act, and its implementing regulations, require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value made to covered recipients, such as physicians and teaching hospitals, and physician ownership and investment interests. Payments made to physicians and research institutions for clinical trials are included within the ambit of this law.

A number of states also have statutes or regulations similar to the federal Anti-Kickback Statute and False Claims Act that apply to items and services reimbursed under Medicaid and other state programs. Some states have anti-kickback statutes that apply to all payors and not just government payors.

Employees

As of February 28, 2013, we had 120 full-time employees, including a total of 52 employees with M.D. or Ph.D. degrees. Of our workforce, 88 employees are engaged in research and development. None of our employees is represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Properties

Our principal facilities consist of approximately 82,798 square feet of research and office space located at 100, 200 and 250 Corporate Court, Middlesex Business Center, South Plainfield, New Jersey, that we occupy under a lease that expires in 2019, with two consecutive five-year renewal options to renew the lease after 2019. We have subleased 11,171 square feet of our space in 250 Corporate Court for a two-year term expiring in October 2014.

Legal proceedings

From time to time in the ordinary course of our business, we are subject to claims, legal proceedings and disputes as a result of patients seeking to participate in our clinical trials or otherwise gain access to our product candidates. These matters are subject to various uncertainties, and it is possible that some of these matters may be resolved unfavorably to us. However, we believe that the ultimate outcome of the matters that are currently pending will not have a material adverse impact on our business.

Management

The following table sets forth the name, age and position of each of our executive officers and directors as of March 15, 2013.

Name	Age	Position
Stuart W. Peltz, Ph.D.	53	Chief Executive Officer and Director
Claudia Hirawat	42	President
Mark E. Boulding	52	Executive Vice President and Chief Legal Officer
Neil Almstead, Ph.D.	46	Senior Vice President, Research and CMC
Jay Barth, M.D.	49	Vice President, Clinical Development
William Hornung	44	Vice President, Finance and Corporate Controller
Michael Schmertzler	61	Chairman of the Board of Directors
Richard Aldrich	58	Director
Axel Bolte	41	Director
Allan Jacobson, Ph.D.	67	Director
Adam Koppel, M.D., Ph.D.	43	Director
Michael Kranda	59	Director
Geoffrey McDonough, M.D.	42	Director
David P. Southwell	52	Director
Jerome B. Zeldis, Ph.D.	62	Director

(1) Member of the Audit Committee

(2) Member of the Compensation Committee

(3) Member of the Nominating and Corporate Governance Committee

Stuart W. Peltz, Ph.D. is a co-founder of our company and has served as our Chief Executive Officer and a member of our board of directors since our inception in 1998. Prior to founding our company, Dr. Peltz was a Professor in the Department of Molecular Genetics & Microbiology at the Robert Wood Johnson Medical School, Rutgers University. Dr. Peltz has published over 80 publications in the area of post-transcriptional control processes and has received a number of scientific awards, including being elected as a Fellow of the American Academy for the Advancement of Science. Dr. Peltz received a Ph.D. from the McArdle Laboratory for Cancer Research at the University of Wisconsin. We believe that Dr. Peltz is qualified to serve on our board of directors because of his extensive executive leadership experience, many years of service as one of our directors and our Chief Executive Officer and extensive knowledge of our company and industry.

Claudia Hirawat has served as our President since April 2012, and previously served as our Senior Vice President, Corporate Development from April 2006 to April 2012 and in other positions since joining PTC in September 2000. Prior to joining PTC, Ms. Hirawat served as a Vice President at LedbetterStevens, a management consulting firm focused on the biopharmaceutical industry, from September 1995 to September 2000.

Mark E. Boulding has served as our Executive Vice President and Chief Legal Officer since March 2012, and previously served as our Senior Vice President and General Counsel from April 2002 to February 2012. Prior to joining us, Mr. Boulding served as General Counsel, Executive Vice President and Secretary of MedicaLogic/Medscape, Inc., a provider of digital health records software and healthcare information, from May 2000 to April 2002. From June 1999 to May 2000, Mr. Boulding served as the General Counsel, Vice President and Secretary of Medscape, Inc., a provider of online health information and education. Mr. Boulding previously was a partner in two Washington, D.C.-based law firms. Mr. Boulding received a J.D. from the University of Michigan and a B.A. from Yale College.

Neil Almstead, Ph.D. has served as our Senior Vice President, Research and CMC since July 2008, and previously served as our Senior Vice President, Chemistry and CMC from January 2007 to June 2008. Prior to joining PTC, Dr. Almstead served as Project Manager at Procter & Gamble Company, a publicly traded consumer products company. Dr. Almstead has co-authored more than 75 publications and patents pertaining to the design and synthesis of lead candidate compounds for genetic disorders, oncology and inflammatory diseases. Dr. Almstead received a B.S. from Clarkson University and a Ph.D. in Organic Chemistry from the University of Illinois at Urbana-Champaign.

Jay Barth, M.D. has served as our Vice President of Clinical Development since January 2011, and previously served as our Executive Director of Clinical Development from February 2009 to December 2010. Prior to joining us, Dr. Barth served as Executive Director of Clinical Research at Merck, a pharmaceutical company, from July 2007 to October 2008. From June 2005 to June 2007, he served as Vice President, Clinical Research and Medical Affairs at Altana Pharma US, Inc., a pharmaceutical company. Dr. Barth received a B.A. from Columbia University and an M.D. from the University of Pennsylvania School of Medicine.

William Hornung has served as our Vice President, Finance since April 2012 and our Corporate Controller since March 2008. Prior to joining PTC, Mr. Hornung served as a senior analyst at Elan Pharmaceuticals, a public biotechnology company from 1998 to March 2002. Mr. Hornung received a B.S. in accounting from William Paterson State University.

Michael Schmertzler has served as a member of our board of directors since August 2001 and as the Chairman of our board of directors since November 2004. From 2008 to 2012, Mr. Schmertzler served as Chief Executive Officer and a director of Kolltan Pharmaceuticals, Inc., a biotechnology company. Since 2001, Mr. Schmertzler has served as a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P., a private equity fund, and the Chair of the investment committee. From 1997 to 2001, Mr. Schmertzler was Co-Head of United States and Canadian Private Equity at Credit Suisse First Boston, an investment banking company. Prior to 1997, Mr. Schmertzler held various management positions with Morgan Stanley and its affiliates, including President of Morgan Stanley Leveraged Capital Funds and head of Morgan Stanley's biotechnology pharmaceuticals group, and was Managing Director and Chief Financial Officer of Lehman Brothers Kuhn Loeb, an investment banking firm. Mr. Schmertzler is currently a director of Lehman Commercial Paper Incorporated, a liquidating post-bankruptcy subsidiary of Lehman Brothers Holdings, Incorporated. Mr. Schmertzler previously served as a director of Cytokinetics, Incorporated. Since 1978, he has been an Adjunct Professor at Yale University. Mr. Schmertzler received a B.A. from Yale College in Molecular Biophysics and Biochemistry, History and City Planning and an M.B.A. from the Harvard Business School. We believe that Mr. Schmertzler is qualified to serve on our board of directors due to his extensive experience as an investment banking and financial professional, his extensive personal knowledge of our industry and his many years of service as one of our directors.

Richard Aldrich has served as a member of our board of directors since March 2013. Mr. Aldrich has served as a partner of Longwood Fund, LP, a venture capital investment fund, since 2010. He founded RA Capital Management LLC, a hedge fund, in 2001 and served as a managing member from 2004 to 2008 and as a co-founding member from 2008 until 2011. He co-founded Sirtris Pharmaceuticals, Inc., which was acquired by GlaxoSmithKline plc in 2008, and served on its board of directors from 2004 to 2008; co-founded Concert Pharmaceuticals, Inc. and has served as Chairman of its board of directors since 2006; co-founded Alnara Pharmaceuticals, Inc., which was acquired by Eli Lilly in 2010, and served on its board of directors from 2008 to 2010; and co-founded OvaScience, Inc. and has served on its board of directors since 2011 and as Chairman of the board since 2012. Mr. Aldrich also joined Vertex Pharmaceuticals, Inc. at its founding in 1989 and served as Senior Vice President and Chief Business Officer until 2001. Mr. Aldrich

also serves on the board of directors of Verastem, Inc., a publicly traded biopharmaceutical company. Mr. Aldrich received a B.S. from Boston College and an M.B.A from the Amos Tuck School at Dartmouth College. We believe that Mr. Aldrich is qualified to serve on our board of directors because of his experience in the life sciences industry and as an entrepreneur and venture capital investors and his service on the boards of directors of other life sciences companies.

Axel Bolte has served as a member of our board of directors since December 2003. Since March 2003, Mr. Bolte has served as investment advisor to HBM Partners AG, a provider of investment advisory services in the life sciences industry. From March 2001 to February 2003, Mr. Bolte was an investment manager of NMT New Medical Technologies AG, a Swiss venture capital company focused on life sciences. Prior to joining NMT New Medical Technologies AG, Mr. Bolte served as a scientist at Serono SA, a biotechnology company. He currently serves or has served on the board of directors of several biotechnology companies, including Newron Pharmaceuticals SpA, Nabriva Therapeutics AG, Opthotech Corporation, MPex Pharmaceuticals, Inc., Lux Biosciences, Inc. and Kolltan Pharmaceuticals, Inc. Mr. Bolte received an M.B.A from the University of St. Gallen, Switzerland and a degree in biochemistry at the Swiss Federal Institute of Technology, Zurich, Switzerland. We believe that Mr. Bolte is qualified to serve on our board of directors because of his many years of service as one of our directors, his extensive experience as a venture capital investor in the life sciences industry and his service on the boards of directors of other life sciences companies.

Allan Jacobson, Ph.D. is a co-founder of our company and has served as a member of our board of directors since our inception in 1998, and previously served as Chairman of our board of directors from 1998 to 2004. Since 2000, Dr. Jacobson has served as Chairman of our scientific advisory board. Since 1994, Dr. Jacobson has been the Chairman of the Department of Microbiology and Physiological Systems at the University of Massachusetts Medical School. In 1982, Dr. Jacobson co-founded Applied bioTechnology, Inc., a biotechnology company, and served as its chairman until its sale in 1991. From 1987 to 1990, Dr. Jacobson served as special limited partner at Euclid Partners, a venture capital firm. Dr. Jacobson received a Ph.D. from Brandeis University in 1971, has authored over 100 publications in the field of post-transcriptional control processes and is an elected member of the American Academy of Microbiology. We believe that Dr. Jacobson is qualified to serve on our board of directors because of his service as one of our directors since our inception, his knowledge of our company and his extensive experience as a founder and leader of new businesses in the life science industry.

Adam Koppel, M.D., Ph.D. has served as a member of our board of directors since March 2013. Since November 2003, Dr. Koppel has served as a Managing Director of Brookside Capital, the public equity affiliate of Bain Capital. Prior to joining Brookside Capital, he was an Associate Principal with McKinsey & Company where he consulted to companies in the pharmaceutical and biotechnology industries. Dr. Koppel received an M.D. and Ph.D. from the University of Pennsylvania School of Medicine, an M.B.A. from the Wharton School of the University of Pennsylvania and a B.A. from Harvard University.

Michael Kranda has served as a member of our board of directors since December 2003. Since September 2006, Mr. Kranda has served as a consultant to Vulcan Capital, the private investment group of Vulcan Inc., and Mr. Kranda served as Managing Director of biotechnology venture investments at Vulcan Capital from September 2003 to September 2006. From July 1996 to July 2002, Mr. Kranda served as Chief Executive Officer at Oxford GlycoSciences, a biotechnology company. Prior to joining Oxford GlycoSciences, Mr. Kranda was President and Chief Operating Officer at Immunex Corporation (now Amgen), a biopharmaceutical company. Mr. Kranda also serves on the board of directors of Cumbre Pharmaceuticals, BiPar Sciences, Nura, Inc., Raven Biotechnologies and the Washington State Biotechnology Business Association. Mr. Kranda received a B.A. and an M.B.A from the University of Washington School of Business. We believe that Mr. Kranda is qualified to serve on our board of directors because of his many

years of service as one of our directors, his extensive experience in the life sciences industry and his service on the boards of directors of other life sciences companies.

Geoffrey McDonough, M.D. has served as a member of our board of directors since November 2012. Since August 2011, Dr. McDonough has served as President and Chief Executive Officer of Swedish Orphan Biovitrum AB (Sobi), a Swedish pharmaceutical company. Prior to joining Sobi, Dr. McDonough held several senior leadership positions at Genzyme Corporation from 2002 to June 2011, including Senior Vice President and General Manager, Personalized Genetic Health, Senior Vice President, Lysosomal Storage Disease (LSD) Therapeutics and most recently, as President of Europe, Middle East and Africa (EMEA). Prior to joining Genzyme, Dr. McDonough co-founded and served as President of Catalyst Medical Solutions, a developer of software for hospital management, and was a practicing internist and pediatrician. Dr. McDonough received a B.A. and a B.Sc. from the University of North Carolina at Chapel Hill and an M.D. from Harvard Medical School. We believe that Dr. McDonough is qualified to serve on our board of directors because of his extensive executive leadership experience and knowledge of our industry.

David P. Southwell has served as a member of our board of directors since December 2005. From March 2010 to September 2012, Mr. Southwell served as the Executive Vice President and Chief Financial Officer, and from 2008 to 2010 served as a member of the board of directors, of Human Genome Sciences, Inc., a biopharmaceutical company. Prior to joining Human Genome Sciences, he served as Executive Vice President and Chief Financial Officer of Sepracor, Inc., a research-based pharmaceutical company, from June 1994 to March 2008, and as Sepracor's Senior Vice President and Chief Financial Officer, from 1994 to 1995. From August 1988 until 1994, Mr. Southwell was associated with Lehman Brothers Inc., a securities firm, in various positions with the investment banking division. Mr. Southwell received a B.A. from Rice University and an M.B.A. from the Tuck School of Business at Dartmouth College. We believe that Mr. Southwell is qualified to serve on our board of directors because of his extensive executive leadership experience and knowledge of our industry.

Jerome B. Zeldis, Ph.D. has served as a member of our board of directors since September 2012. Dr. Zeldis currently serves as the Chief Executive Officer of Celgene Global Health and the Chief Medical Officer of Celgene Corporation, a public biopharmaceutical company, where he has been employed since 1997. He previously served as Celgene's Senior Vice President of Clinical Research and Medical Affairs. Previously, Dr. Zeldis served as Assistant Professor of Medicine at Harvard Medical School, Associate Professor of Medicine at University of California, Davis, Clinical Associate Professor of Medicine at Cornell Medical School and Professor of Clinical Medicine at the Robert Wood Johnson Medical School. Dr. Zeldis received an A.B. and M.S. from Brown University and an M.Phil., M.D. and Ph.D. in Molecular Biophysics and Biochemistry (immunochemistry) from Yale University. Dr. Zeldis has served on the board of directors of Soligenix, Inc., a public biopharmaceutical company, since June 2011, and on the board of directors of Alliqua, Inc., a public biomedical company, since May 2012. We believe that Dr. Zeldis is qualified to serve on our board of directors because of his extensive executive leadership experience and knowledge of our industry.

Board composition and election of directors

Our board of directors is currently authorized to have 11 members. Our board of directors is divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. The members of the classes are divided as follows:

- the class I directors are , and their term expires at our annual meeting of stockholders to be held in 2014;
- the class II directors are , and their term expires at our annual meeting of stockholders to be held in 2015; and

- the class III directors are , and their term expires at our annual meeting of stockholders to be held in 2016.

Upon the expiration of the term of a class of directors, directors in that class are eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires. In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our directors may be removed only for cause by the affirmative vote of the holders of 75% or more of our voting stock.

Under applicable NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of our board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. Our board of directors has determined that all of our directors, other than Dr. Peltz, are independent directors, as defined by the applicable NASDAQ Rules. In making such determination, the board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that the board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director.

There are no family relationships among any of our directors or executive officers.

Board committees

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee, each of which will operate, upon the closing of this offering, under a charter that has been approved by our board. The composition of each committee will be effective upon the closing of this offering.

Our board of directors has determined that all of the members of the audit committee, the compensation committee and the nominating and corporate governance committee are independent as defined under the NASDAQ Rules, including, in the case of all the members of our audit committee, the independence requirements contemplated by Rule 10A-3 under the Exchange Act.

Audit committee

The members of our audit committee are . chairs the audit committee. Upon the closing of this offering, our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function;
- overseeing our risk assessment and risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, our independent registered public accounting firm and management;

- reviewing and approving or ratifying any related person transactions; and
- preparing the audit committee report required by Securities and Exchange Commission, or SEC, rules.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that i s an "audit committee financial expert" as defined in applicable SEC rules.

Compensation committee

The members of our compensation committee are . chairs the compensation committee. Upon the closing of this offering, our compensation committee's responsibilities will include:

- reviewing and approving, or making recommendations to our board with respect to, the compensation of our chief executive officer and our other executive officers;
- overseeing an evaluation of our senior executives;
- overseeing and administering our cash and equity incentive plans;
- reviewing and making recommendations to our board with respect to director compensation;
- reviewing and discussing annually with management our compensation disclosure required by SEC rules; and
- preparing the compensation committee report required by SEC rules.

Nominating and corporate governance committee

The members of our nominating and corporate governance committee are . chairs the nominating and corporate governance committee. Upon the closing of this offering, our nominating and corporate governance committee's responsibilities will include:

- identifying individuals qualified to become members of our board;
- recommending to our board the persons to be nominated for election as directors and to each of our board's committees;
- reviewing and making recommendations to our board with respect to our board leadership structure;
- reviewing and making recommendations to our board with respect to management succession planning;
- developing and recommending to our board corporate governance principles; and
- overseeing a periodic evaluation of our board.

Compensation committee interlocks and insider participation

None of our executive officers serves as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. None of the members of our compensation committee is, or has ever been, an officer or employee of our company.

Executive compensation

This section describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers in 2012. Our "named executive officers" for 2012 are Stuart W. Peltz, our Chief Executive Officer, Claudia Hirawat, our President, and Jay Barth, our Vice President, Clinical Development. This section also provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our executive officers and is intended to place in perspective the data presented in the tables and narrative that follow.

Summary compensation table

The following table sets forth information regarding compensation awarded to, earned by or paid to our named executive officers during 2012.

Name and principal position	Year	Salary ($)	Bonus ($)	Option awards ($)(1)	Non-equity incentive plan compensation ($)(2)	All other compensation ($)(3)	Total ($)
Stuart W. Peltz, Ph.D.(4) *Chief Executive Officer*	2012	446,250	—	67,175	—	4,362	517,787
Claudia Hirawat *President*	2012	281,039	—	30,900	—	56,668	368,607
Jay Barth, M.D. *Vice President, Clinical Development*	2012	325,000	40,000(5)	21,496	—	3,112	389,608

(1) The amounts reported in the "Option awards" column reflect the aggregate fair value of share-based compensation awarded during the year computed in accordance with the provisions of Financial Accounting Standards Board Accounting Standard Codification, or ASC, Topic 718. See Note 2 to our financial statements appearing at the end of this prospectus regarding assumptions underlying the valuation of equity awards.

(2) The amounts reported in the "Non-equity incentive plan compensation" column represents awards to our named executive officers under our annual cash bonus program. Annual cash bonus compensation for 2012 will be determined in 2013.

(3) The amounts reported in the "All other compensation" column reflect, for each named executive officer, the sum of the incremental cost to us of all perquisites and other personal benefits and are comprised of (i) the amount we contributed to our 401(k) plan in respect of such executive officer; (ii) the dollar value of medical and life insurance premiums paid by us on behalf of each of the named executive officers; and (iii) with respect to Ms. Hirawat, an income tax gross-up amount of $53,593.

(4) Dr. Peltz also serves a member of our board of directors but does not receive any additional compensation for his service as a director.

(5) We granted Dr. Barth a retention bonus of $40,000 in March 2012.

In 2012, we paid base salaries of $446,250 to Dr. Peltz, $281,039 to Ms. Hirawat and $325,000 to Dr. Barth. Base salaries are used to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incents our executive officers to remain in our employment during the vesting period. Accordingly, our board of

directors periodically reviews the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them in the form of stock options. In 2012, based upon our overall performance, we granted to Dr. Peltz an option to purchase 416 shares of our common stock, to Ms. Hirawat an option to purchase 191 shares of our common stock and to Dr. Barth an option to purchase 133 shares of our common stock.

Outstanding option awards at December 31, 2012

The following table sets forth information regarding outstanding stock options held by our named executive officers as of December 31, 2012:

Name	Number of securities underlying unexercised options exercisable (#)	Number of securities underlying unexercised options unexercisable (#)	Option exercise price ($/share)	Option expiration date
Stuart W. Peltz, Ph.D.	5,649	—	226.80	11/5/2014
Chief Executive Officer	144	—	226.80	5/25/2015
	136	—	392.40	3/1/2016
	323	—	626.40	4/18/2017
	708	—	735.60	1/25/2018
	294	—	735.60	4/1/2018
	546	36(1)	451.20	5/15/2019
	401	182(2)	1,149.60	2/2/2020
	237	304(3)	490.80	4/27/2021
	—	416(4)	218.40	1/10/2022
Claudia Hirawat	890	—	226.80	11/5/2014
President	106	—	226.80	5/24/2015
	136	—	392.40	3/1/2016
	250	—	626.40	4/18/2017
	166	—	735.60	1/25/2018
	195	—	735.60	4/1/2018
	218	14(1)	451.20	5/15/2019
	137	62(2)	1,149.60	2/2/2020
	102	131(3)	490.80	4/27/2021
	—	191(4)	218.40	1/10/2022
Jay Barth, M.D.	125	41(1)	451.20	5/15/2019
Vice President, Clinical	43	19(2)	1,149.60	2/2/2020
Development	80	103(3)	490.80	4/27/2021
	—	133(4)	218.40	1/10/2022

(1) This option vests over four years, with 25% of the shares underlying the option vested on January 1, 2010 and 6.25% of the shares underlying the option vesting quarterly thereafter.

(2) This option vests over four years, with 25% of the shares underlying the option vested on January 1, 2011 and 6.25% of the shares underlying the option vesting quarterly thereafter.

(3) This option vests over four years, with 25% of the shares underlying the option vested on January 1, 2012, the first anniversary of the date of grant, and 6.25% of the shares underlying the option vesting quarterly thereafter.

(4) This option vests over four years, with 25% of the shares underlying the option vested on January 1, 2013, the first anniversary of the date of grant, and 6.25% of the shares underlying the option vesting quarterly thereafter.

In March 2013, our board of directors granted restricted stock awards to our executive officers, including our named executive officers, pursuant to our 2013 stock incentive plan as follows:

Name	Stock award (#)	Grant date fair value ($)(1)
Stuart W. Peltz, Ph.D.	188,803	
Claudia Hirawat	50,057	
Jay Barth, M.D.	7,387	
Mark E. Boulding	42,284	
Neil Almstead, Ph.D.	40,740	
William Hornung	4,640	

(1) See Note 2 to the financial statements included at the end of this prospectus regarding assumptions underlying the valuation of equity awards.

Stock option and other compensation plans

The four equity incentive plans described in this section are the 1998 employee, director and consultant stock option plan, as amended and restated, or the 1998 plan, the 2009 equity and long term incentive plan, or the 2009 plan, the 2013 stock incentive plan, or the 2013 plan, and the 2013 public company stock incentive plan, or the 2013 public company plan. Prior to this offering, we granted awards to eligible participants under the 1998 plan, the 2009 plan and the 2013 plan. Following the closing of this offering, we expect to grant awards to eligible participants under the 2013 public company plan.

1998 employee, director and consultant stock option plan

The 1998 plan was adopted by our board of directors and approved by our stockholders. The 1998 plan provides for the grant of incentive stock options and non-statutory stock options. A maximum of 33,133 shares of our common stock are authorized for issuance under the 1998 plan.

In accordance with the terms of the 1998 plan, our board of directors has authorized our compensation committee to administer the plan.

The terms of awards under the 1998 plan are set forth in the applicable option certificates.

Under the 1998 plan, in the event of a stock dividend or stock split, the number of shares of common stock deliverable upon the exercise of options may be proportionately increased or decreased, with appropriate adjustments to the applicable exercise price.

If a merger of our company or similar corporate event occurs, the administrator of the plan or the board of directors of the successor corporation, in its discretion, shall:

- provide that the outstanding options under the 1998 plan be assumed or substituted by the successor corporation;
- upon written notice to optionees, provide that all unexercised options will terminate, unless exercised, immediately prior to the consummation of such transaction; or
- provide that all or any of the outstanding options will terminate in exchange for a cash payment equal to their value.

In the event of a recapitalization or other reorganization where securities of the company or another corporation are issued with respect to the outstanding shares of common stock, options are exercisable for

securities that would have been received if such options had been exercised prior to such recapitalization or reorganization.

As of March 15, 2013, there were options to purchase 23,798 shares of our common stock outstanding under the 1998 plan, at a weighted-average exercise price of $400.90 per share, and options to purchase 1,057 shares of our common stock had been exercised. In August 2008, the 1998 plan expired and since then no further grants of stock options have been made under this plan. All shares available to grant under the 1998 plan automatically transferred to the 2009 plan at that time.

2009 equity and long term incentive plan

The 2009 plan was adopted by our board of directors in February 2009 and approved by our stockholders in March 2009. The 2009 plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights and other stock-based and cash-based awards. Our employees, officers, directors, consultants and advisors are eligible to receive awards under our 2009 plan; however, incentive stock options may only be granted to our employees. Our board of directors administers the 2009 plan.

The maximum number of shares of our common stock with respect to which awards may be granted to any participant under the 2009 plan is 3,333 per year. No award may be granted under the 2009 stock plan after May 8, 2019, but the vesting and effectiveness of awards granted before that date may extend beyond that date. Our board of directors may amend, suspend or terminate the 2009 stock plan at any time, subject to applicable law or stock market requirements.

The 2009 plan provides that the number of shares of our common stock reserved for issuance under the plan is 6,666 shares (the initial number of shares provided to the plan), *plus* an annual evergreen equal to the lowest of 6,666 shares of our common stock, 4% of our shares of common stock outstanding on January 1 of each year (including all shares of common stock issuable upon conversion of shares of our preferred stock) and an amount determined by our board of directors, *plus* the sum (up to a maximum of 32,083 shares) of any shares of our common stock reserved for issuance under the 1998 plan and any shares of our common stock that have expired or terminated, or are otherwise surrendered, canceled, forfeited or repurchased by the company, pursuant to the 1998 plan.

Upon the occurrence of a fundamental event, as defined in the 2009 plan, our board of directors shall provide that all outstanding stock options be assumed, or substantially equivalent awards be substituted, by the acquiring or successor corporation (or an affiliate thereof). Restricted stock awards generally remain unchanged. Upon a change in control event, as defined in the 2009 plan, then, with respect to any unvested stock option, or restricted stock award, as applicable, one-half of the number of unvested shares subject to such option or restricted stock award, as applicable, shall vest and become immediately exercisable upon the occurrence of such change in control event and all remaining unvested shares subject to such option or restricted stock award, as applicable, shall continue to vest accordance with the original vesting schedule, provided that all such unvested shares shall become immediately vested and/or exercisable in full if, on or prior to the first anniversary of the date of the consummation of the change in control event, the participant's employment with us or the acquiring or succeeding corporation is terminated for good reason by the participant or without good cause by the company or acquiring or succeeding corporation (in each case as defined in the 2009 plan).

In the event the acquiring or successor corporation does not agree to assume, or substitute for, options granted under the 2009 plan, or in the event of a liquidation or dissolution of the company, our board of directors will provide that all unexercised options will become exercisable in full as of a specified time prior to the fundamental event and will terminate upon the consummation of such fundamental event

unless exercised by the participant. In the event of a fundamental event where holders of our common stock will receive a cash payment for surrendering such shares, then our board of directors may instead provide that all outstanding options will terminate upon the consummation of such fundamental event and that each participant will receive a cash payment equal to the value of the participant's options.

In the event of a stock dividend, stock split or similar event, our board of directors will equitably adjust the terms of awards granted under the 2009 plan.

Our board of directors may at any time provide that any award will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part, as the case may be.

As of March 15, 2013, there were options to purchase an aggregate of 18,227 shares of common stock outstanding under the 2009 plan, at a weighted average exercise price of $559.87 per share, and an aggregate of 13 shares of common stock issued upon the exercise of options granted under the 2009 plan. If the 2013 public company plan is approved by our stockholders, we will grant no further stock options or other awards under the 2009 plan. However, any shares of common stock reserved for issuance under the 2009 plan that remain available for issuance and any shares of common stock subject to awards under the 2009 plan that expire, terminate, or are otherwise surrendered, canceled, forfeited or repurchased without having been fully exercised or resulting in any common stock being issued shall be available for grant under the 2013 public company plan up to a specified number of shares.

2013 stock incentive plan

The 2013 plan was adopted by our board of directors and approved by our stockholders on March 5, 2013. The 2013 plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. A maximum of 739,937 shares of our common stock are authorized for issuance under the 2013 plan. Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2013 plan.

Pursuant to the terms of the 2013 plan, our board of directors administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;
- the duration of options, which may not be in excess of ten years;
- the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and
- the number of shares of our common stock subject to and the terms of any stock appreciation rights, restricted stock awards, restricted stock units or other stock-based awards and the terms and conditions of such awards, including conditions for repurchase, issue price and repurchase price.

Upon a merger or other reorganization event, our board of directors may, in its sole discretion, take any one or more of the following actions pursuant to the 2013 plan as to some or all outstanding awards other than restricted stock:

- provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or successor corporation (or an affiliate thereof);
- upon written notice to a participant, provide that all of the participant's unexercised awards will terminate immediately prior to the consummation of such reorganization event unless exercised by the participant;
- provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;
- in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award; and/or
- provide that, in connection with a liquidation or dissolution, awards shall convert into the right to receive liquidation proceeds.

Our board of directors does not need to take the same action with respect to all awards and may take different actions with respect to portions of the same award.

In the case of certain restricted stock units, where no assumption or substitution is permitted, the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights with respect to outstanding restricted stock awards will continue for the benefit of the successor company and will, unless the board of directors may otherwise determine, apply to the cash, securities or other property into which shares of our common stock are converted or exchanged pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award.

At any time, our board of directors may, in its sole discretion, provide that any award under the 2013 plan will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part.

No award may be granted under the 2013 plan on or after March 5, 2023. Our board of directors may amend, suspend or terminate the 2013 plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

As of March 15, 2013, options to purchase 4,613 shares of our common stock and 735,324 shares of restricted common stock were outstanding under the 2013 plan. If the 2013 public company plan is approved by our stockholders, we will grant no further stock options or other awards under the 2013 plan. However, any shares of common stock subject to awards under the 2013 plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased without having been fully exercised or resulting in any common stock being issued shall be available for grant under the 2013 public company plan up to a specified number of shares.

2013 public company stock incentive plan

We expect our board of directors to adopt and our stockholders to approve the 2013 public company plan, which will become effective immediately prior to the closing of this offering. The 2013 public company plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. Upon effectiveness of the 2013 public company plan, the number of shares of our common stock that will be reserved for issuance under the 2013 public company plan will be the sum of (1) shares plus (2) the number of shares (up to shares) equal to the sum of the number of shares of our common stock then available for issuance under the 2009 plan and the number of shares of our common stock subject to outstanding awards under the 2009 plan and the 2013 plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of and each subsequent anniversary until the expiration of the 2013 public company plan, equal to the lowest of shares of our common stock, % of the number of shares of our common stock outstanding on the first day of the fiscal year and an amount determined by our board of directors.

Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2013 public company plan. However, incentive stock options may only be granted to our employees. The maximum number of shares of our common stock with respect to which awards may be granted to any participant under the 2013 public company plan is per calendar year. For purposes of this limit on the maximum number of shares that may be awarded to any participant, the combination of an option in tandem with a stock appreciation right will be treated as a single award.

Pursuant to the terms of the 2013 public company plan, our board of directors administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;
- the duration of options, which may not be in excess of ten years;
- the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and
- the number of shares of our common stock subject to and the terms of any stock appreciation rights, restricted stock awards, restricted stock units or other stock-based awards and the terms and conditions of such awards, including conditions for repurchase, issue price and repurchase price.

If our board of directors delegates authority to an executive officer to grant awards under the 2013 public company plan, the executive officer has the power to make awards to all of our employees, except

executive officers. Our board of directors will fix the terms of the awards to be granted by such executive officer, including the exercise price of such awards, and the maximum number of shares subject to awards that such executive officer may make.

Upon a merger or other reorganization event, our board of directors may, in its sole discretion, take any one or more of the following actions pursuant to the 2013 public company plan as to some or all outstanding awards other than restricted stock:

- provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or successor corporation (or an affiliate thereof);
- upon written notice to a participant, provide that all of the participant's unexercised awards will terminate immediately prior to the consummation of such reorganization event unless exercised by the participant;
- provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;
- in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award; and/or
- provide that, in connection with a liquidation or dissolution, awards shall convert into the right to receive liquidation proceeds.

Our board of directors does not need to take the same action with respect to all awards and may take different actions with respect to portions of the same award.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights with respect to outstanding restricted stock awards will continue for the benefit of the successor company and will, unless the board of directors may otherwise determine, apply to the cash, securities or other property into which shares of our common stock are converted or exchanged pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award.

At any time, our board of directors may, in its sole discretion, provide that any award under the 2013 public company plan will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part.

No award may be granted under the 2013 public company plan on or after , 2023. Our board of directors may amend, suspend or terminate the 2013 public company plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

401(k) retirement plan

We maintain a 401(k) retirement plan that is intended to be a tax-qualified defined contribution plan under Section 401(k) of the Internal Revenue Code. In general, all of our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes a salary deferral arrangement pursuant to which participants may elect to reduce their current compensation by up to the statutorily prescribed limit, equal to $17,500 in 2013, and have the amount of the reduction contributed to the 401(k) plan.

Limitations on liability and indemnification

Our certificate of incorporation, which will become effective upon the closing of this offering, limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty or other duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- for voting for or assenting to unlawful payments of dividends, stock repurchases or other distributions; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the Delaware General Corporation Law is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the Delaware General Corporation Law.

In addition, our certificate of incorporation, which will become effective upon the closing of this offering, provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we have entered into indemnification agreements with our directors. These indemnification agreements may require us, among other things, to indemnify each such director for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him in any action or proceeding arising out of his service as one of our directors.

Certain of our non-employee directors may, through their relationships with their employers, be insured and/or indemnified against certain liabilities incurred in their capacity as members of our board of directors.

Rule 10b5-1 sales plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. The director or officer may amend or terminate the plan in some circumstances. Our directors and executive officers may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

Director compensation

Dr. Peltz, one of our directors who also serves as our Chief Executive Officer, does not receive any additional compensation for his service as a director.

Our non-employee directors are compensated for their services on our board of directors as follows:

- each non-employee director receives an option to purchase 1,875 shares of our common stock upon his or her initial election or appointment to our board of directors;
- each non-employee director receives an annual retainer of an option to purchase 83 shares of our common stock; and
- each non-employee directors who serves as chairman of the board of directors, chairman of a committee of the board or a member of a committee of the board, receives additional equity compensation as follows:
    - chairman of the board—an additional annual stock option to purchase shares of our common stock having a value on the date of grant of $25,000;
    - chairman of the audit committee, compensation committee or the nominating and corporate governance committee—with respect to each such position, an additional annual stock option to purchase shares of our common stock having a value on the date of grant of $5,000; and
    - member of the audit committee, compensation committee or the nominating and corporate governance committee—with respect to each such membership, an additional annual stock option to purchase shares of our common stock having a value on the date of grant of $2,500.

The stock options granted to our non-employee directors have an exercise price equal to the fair market value of our common stock on the date of grant, expire ten years after the date of grant, subject to the director's continued service on our board, and are exercisable quarterly over a three year period from the date of grant.

Each member of our board of directors is also entitled to be reimbursed for reasonable travel and other expenses incurred in connection with attending meetings of the board of directors and any committee on which he or she serves.

For the year ended December 31, 2012, the only compensation we awarded or paid to our directors, other than Dr. Peltz, were stock option awards, as set forth in the following table.

Name	Option award ($)(1)
Michael Schmertzler	44,488
Axel Bolte	16,348
Soren Carlsen, Ph.D.(2)	—
Allan Jacobson, Ph.D.	13,400
Michael Kranda	4,874
Geoffrey McDonough, M.D.	—
Deepa Pakianathan, Ph.D.(2)	13,400
David Southwell	17,822
Peter Svennilson(2)	21,574
Jerome Zeldis, Ph.D.	—
Carl Goldfischer, M.D.	17,882

(1) The amounts reported in this column represent the aggregate grant date fair value of the options granted to our non-employee directors during 2012 computed in accordance with ASC 718. See Note 2 to the financial statements included at the end of this prospectus regarding assumptions underlying the valuation of equity awards.

(2) Drs. Carlsen and Pakianathan and Mr. Svennilson resigned from our board of directors on March 7, 2013.

In March 2013, our board of directors granted restricted stock awards and stock options to certain of our directors, pursuant to our 2013 stock incentive plan as follows:

Name	Stock award (#)(1)	Option award (#)(2)	Grant date fair value ($)(3)
Deepa Pakianathan, Ph.D.(4)	—	3,637	
Allan Jacobson, Ph.D.	38,080	—	
Michael Kranda	5,928	—	
Geoffrey McDonough, M.D.	909	—	
Michael Schmertzler	26,766	—	
David Southwell	13,238	—	
Peter Svennilson(4)	—	976	

(1) The restricted stock reported in this column vests as to 50% of the award on each of the first and second anniversary of the date of grant.

(2) The options reported in this column were fully vested on the date of grant.

(3) See Note 2 to the financial statements included at the end of this prospectus regarding assumptions underlying the valuation of equity awards.

(4) Dr. Pakianathan and Mr. Svennilson resigned from our board of directors on March 7, 2013.

Our board of directors intends to approve a compensation policy for our non-employee directors that will become effective upon the closing of this offering. This policy will be intended to provide a total compensation package that enables us to attract and retain qualified and experienced individuals to serve as directors and to align our directors' interests with those of our stockholders.

Transactions with related persons

Since January 1, 2010, we have engaged in the following transactions with our directors, executive officers and holders of more than 5% of our voting securities, and affiliates of our directors, executive officers and holders of more than 5% of our voting securities. We believe that all of these transactions were on terms as favorable as could have been obtained from unrelated third parties.

Series four senior preferred stock financing and reclassification of outstanding preferred stock

In March 2013, we issued and sold an aggregate of 4,497,035 shares of our series four senior preferred stock, at a price per share of $12.00, for an aggregate purchase price of $53,964,420. In addition, we issued an aggregate of 502,919 shares of our series four senior preferred stock upon conversion of the convertible promissory notes described below that we originally issued in January and February 2013. The following table sets forth the number of shares of our series four senior preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the number of shares of our series four senior preferred stock that we issued to our directors, executive officers and 5% stockholders and their affiliates upon conversion of the convertible promissory notes described below.

Name	Shares of series four senior preferred stock purchased	Shares of series four senior preferred stock issued upon conversion of convertible promissory notes
Credit Suisse First Boston Equity Partners, L.P. and affiliates(1)	165,464	111,389
HBM Healthcare Investments (Cayman) Ltd.(2)	187,220	92,958
Vulcan Ventures Incorporated and affiliates(3)	114,608	68,725
Brookside Capital Partners Fund, L.P.(4)	1,083,333	—
Celgene European Investment Company LLC and affiliate(5)	76,915	51,778
Delphi Ventures and affiliates(6)	—	50,058
Section Six Partners, L.P.(7)	103,819	51,547

(1) Consists of (i) 129,220 shares purchased and 86,989 shares issued upon conversion of convertible promissory notes held by Credit Suisse First Boston Equity Partners, L.P.; (ii) 36,120 shares purchased and 24,316 shares issued upon conversion of convertible promissory notes held by Credit Suisse First Boston Equity Partners (Bermuda), L.P.; and (iii) 124 shares purchased and 84 shares issued upon conversion of convertible promissory notes held by Credit Suisse First Boston U.S. Executive Advisors, L.P. Mr. Schmertzler, a member of our board of directors, is a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P. and affiliates. Mr. Schmertzler disclaims beneficial ownership of the shares held by entities affiliated with Credit Suisse Credit Suisse First Boston Equity Partners, L.P. and affiliates except to the extent of any pecuniary interest therein.

(2) The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investments (Cayman) Ltd. is comprised of John Arnold, Richard Coles, Sophia Harris, Dr. Andreas Wicki and John Urquhart, none of whom has individual voting or investment power with respect to such shares, and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Mr. Bolte, a member of our board of directors, is an advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Mr. Bolte has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd., and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(3) Consists of shares held by VCVC III LLC. Mr. Kranda, a member of our board of directors, is a consultant to Vulcan Capital Venture Capital I LLC. VCVC III LLC and Vulcan Capital Venture Capital I LLC are each controlled by entities owned by Paul Allen. Mr. Kranda does not have voting or investment power over the shares held by Vulcan Ventures Incorporated and its affiliates, and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(4) Dr. Koppel, a member of our board of directors, is a Managing Director of Brookside Capital, LLC, the investment advisor to Brookside Capital Partners Fund, L.P., Dr. Koppel disclaims beneficial ownership of the shares held by Brookside Capital Partners Fund, L.P. except to the extent of any pecuniary interest therein.

(5) Consists of (i) 76,915 shares purchased by Celgene European Investment Company LLC and (ii) 51,778 shares issued upon conversion of convertible promissory notes held by Celgene Corporation. Dr. Zeldis, a member of our board of directors, is an employee of Celgene

Corporation. Celgene European Investment Company LLC is a wholly owned subsidiary of Celgene Corporation. Dr. Zeldis disclaims beneficial ownership of the shares held by Celgene European Investment Company LLC and Celgene Corporation except to the extent of any pecuniary interest therein.

(6) Consists of (i) 31,306 shares issued to Delphi Ventures V, L.P.; (ii) 339 shares issued to Delphi BioInvestments V, L.P.; (iii) 9,115 shares issued to Delphi Ventures VII, L.P.; (iv) 91 shares issued to Delphi BioInvestments VII, L.P.; (v) 9,118 shares issued to Delphi Ventures VIII, L.P.; and (vi) 89 shares issued to Delphi BioInvestments VIII, L.P.

(7) Mr. Schmertzler is a general and limited partner of, and trustee of certain family trusts holding interests in, Section Six Partners, L.P., and disclaims beneficial ownership of the shares held by Section Six Partners, L.P. except to the extent of any pecuniary interest therein.

In connection with the series four senior preferred stock financing, we effected a one-for-120 reverse stock split of our common stock and a reclassification of our previously outstanding preferred stock into an aggregate of 6,700,487 shares of series five junior preferred stock. In addition, we issued an aggregate of 2,095,515 shares of our series five junior preferred stock upon the automatic exercise of the preferred stock warrants described below that we originally issued in January 2013. The following table sets forth the number of shares of our series five junior preferred stock that we issued to our directors, executive officers and 5% stockholders and their affiliates upon the reclassification of our previously outstanding preferred stock and the number of shares of our series five junior preferred stock that we issued to our directors, executive officers and 5% stockholders and their affiliates upon the automatic exercise of the preferred stock warrants described below.

Name	Shares of series five junior preferred stock issued upon reclassification of outstanding preferred stock	Shares of series five junior preferred stock issued upon automatic exercise of preferred stock warrants
Credit Suisse First Boston Equity Partners, L.P. and affiliates(1)	1,515,800	464,229
HBM Healthcare Investments (Cayman) Ltd.(2)	1,173,898	387,419
Vulcan Ventures Incorporated and affiliates(3)	898,664	286,401
Celgene European Investment Company LLC and affiliate(4)	726,725	215,794
Delphi Ventures and affiliates(5)	697,108	208,600
Section Six Partners, L.P.(6)	394,166	214,504

(1) Consists of (i) 1,180,858 shares issued upon reclassification and 362,542 shares issued upon automatic exercise held by Credit Suisse First Boston Equity Partners, L.P.; (ii) 330,081 shares issued upon reclassification and 101,338 shares issued upon automatic exercise held by Credit Suisse First Boston Equity Partners (Bermuda), L.P.; (iii) 1,140 shares issued upon reclassification and 349 shares issued upon automatic exercise held by Credit Suisse First Boston U.S. Executive Advisors, L.P.; (iv) 234 shares issued upon automatic exercise held by Credit Suisse First Boston Finders & Screeners LP; and (v) 3,487 shares issued upon reclassification held by EMA Private Equity Fund 1999, LP. Mr. Schmertzler, a member of our board of directors, is a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P. and affiliates. Mr. Schmertzler disclaims beneficial ownership of the shares held by entities affiliated with Credit Suisse Credit Suisse First Boston Equity Partners, L.P. and affiliates except to the extent of any pecuniary interest therein.

(2) The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investment (Cayman) Ltd. is comprised of John Arnold, Richard Coles, Sophia Harris, Dr. Andreas Wicki and John Urquhart, none of whom has individual voting or investment power with respect to such shares, and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Mr. Bolte, a member of our board of directors, is an advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Neither HBM Partners AG nor Mr. Bolte has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd., and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(3) Consists of 101,562 shares issued upon reclassification held by Vulcan Ventures Incorporated; (ii) 797,102 shares issued upon reclassification held by Vulcan Capital Venture Capital I LLC; and (iii) 286,401 shares issued upon automatic exercise held by VCVC III LLC. Mr. Kranda, a member of our board of directors, is a consultant to Vulcan Capital Venture Capital I LLC. Vulcan Capital Venture Capital I LLC, VCVC III LLC, and Vulcan Ventures Incorporated are each controlled by entities owned by Paul Allen. Mr. Kranda does not have voting or investment power over the shares held by Vulcan Ventures Incorporated and its affiliates, and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(4) Consists of (i) 726,725 shares issued upon reclassification held by Celgene European Investment Company LLC and (ii) 215,794 shares issued upon automatic exercise held by Celgene Corporation. Dr. Zeldis, a member of our board of directors, is an employee of Celgene Corporation. Celgene European Investment Company LLC is a wholly owned subsidiary of Celgene Corporation. Dr. Zeldis disclaims beneficial ownership of the shares held by Celgene European Investment Company LLC and Celgene Corporation except to the extent of any pecuniary interest therein.

(5) Consists of (i) 412,965 shares issued upon reclassification and 130,465 shares issued upon automatic exercise held by Delphi Ventures V, L.P.; (ii) 4,475 shares issued upon reclassification and 1,411 shares issued upon automatic exercise held by Delphi BioInvestments V, L.P.; (iii) 138,448 shares issued upon reclassification and 37,982 shares issued upon automatic exercise held by Delphi Ventures VII, L.P.; (iv) 1,384 shares issued upon reclassification and 378 shares issued upon automatic exercise held by Delphi BioInvestments VII, L.P.; (v) 138,483 shares issued upon reclassification and 37,994 shares issued upon automatic exercise held by Delphi Ventures VIII, L.P.; and (vi) 1,353 shares issued upon reclassification and 370 shares issued upon automatic exercise held by Delphi BioInvestments VIII, L.P.

(6) Mr. Schmertzler is a general and limited partner of, and trustee of certain family trusts holding interests in, Section Six Partners, L.P., and disclaims beneficial ownership of the shares held by Section Six Partners, L.P. except to the extent of any pecuniary interest therein.

Bridge financing

In January and February 2013, we issued convertible promissory notes in an aggregate principal amount of $6,000,000. In connection with this bridge financing, we also issued to the holders of the promissory notes warrants to purchase an aggregate of 515,186 shares of our series one preferred stock, at an exercise price of $0.01 per share, and warrants to purchase an aggregate of 2,012,489 shares of our series two preferred stock, an exercise price of $0.01 per share. The following table sets forth the principal amount of the promissory notes and the number of series two warrants and series three warrants that we issued to our directors, executive officers and 5% stockholders and their affiliates.

Name	Aggregate principal amount of promissory notes	Warrants to purchase shares of series one preferred stock(1)	Warrants to purchase shares of series two preferred stock(1)
Credit Suisse First Boston Equity Partners, L.P. and affiliates(2)	$ 1,329,182	114,131	445,828
HBM Healthcare Investments (Cayman) Ltd.(3)	1,109,250	95,247	372,061
Vulcan Ventures Incorporated and affiliates(4)	820,021	70,412	275,048
Celgene Corporation(5)	617,860	53,053	207,240
Delphi Ventures and affiliates(6)	597,299	51,285	200,342
Section Six Partners, L.P.(7)	614,172	52,736	206,003

(1) In connection with the recapitalization and reverse stock split described above, the warrants described in the table above were automatically adjusted to be exercisable into shares of our series five preferred stock at the applicable conversion ratio.

(2) Consists of (i) $1,038,024 principal amount of notes, 89,131 series one warrants, and 348,170 series two warrants held by Credit Suisse First Boston Equity Partners, L.P.; (ii) $290,154 principal amount of notes, 24,914 series one warrants, and 97,322 series two warrants held by Credit Suisse First Boston Equity Partners (Bermuda), L.P.; and (iii) $1,004 principal amount of notes, 86 series one warrants, and 336 series two warrants held by Credit Suisse First Boston U.S. Executive Advisors, L.P. Mr. Schmertzler, a member of our board of directors, is a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P. and affiliates, who disclaims beneficial ownership of the shares held by entities affiliated with Credit Suisse Credit Suisse First Boston Equity Partners, L.P. and affiliates except to the extent of any pecuniary interest therein.

(3) The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investments (Cayman) Ltd. is comprised of John Arnold, Richard Coles, Sophia Harris, Dr. Andreas Wicki and John Urquhart, none of whom has individual voting or investment power with respect to such shares, and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Mr. Bolte, a member of our board of directors, is an advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Mr. Bolte has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd., and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(4) Consists of notes and warrants held by VCVC III LLC. Mr. Kranda, a member of our board of directors, is a consultant to Vulcan Capital Venture Capital I LLC. VCVC III LLC and Vulcan Capital Venture Capital I LLC controlled by entities owned by Paul Allen. Mr. Kranda does not have voting or investment power over the shares held by Vulcan Ventures Incorporated and its affiliates, and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(5) Dr. Zeldis, a member of our board of directors, is an employee of Celgene Corporation. Celgene European Investment Company LLC is a wholly owned subsidiary of Celgene Corporation. Dr. Zeldis disclaims beneficial ownership of the shares held by Celgene Corporation except to the extent of any pecuniary interest therein.

(6) Consists of (i) $373,548 principal amount of notes, 32,075 series one warrants, and 125,294 series two warrants held by Delphi Ventures V, L.P.; (ii) $4,050 principal amount of notes, 347 series one warrants, and 1,358 series two warrants held by Delphi BioInvestments V, L.P.; (iii) $108,761 principal amount of notes, 9,338 series one warrants, and 36,480 series two warrants held by Delphi Ventures VII, L.P.; (iv) $1,088 principal amount of notes, 93 series one warrants, and 364 series two warrants held by Delphi BioInvestments VII, L.P.; (v) $108,790 principal amount of notes, 9,341 series one warrants, and 36,490 series two warrants held by Delphi Ventures VIII, L.P.; and (vi) $1,062 principal amount of notes, 91 series one warrants, and 356 series two warrants held by Delphi BioInvestments VIII, L.P.

(7) Mr. Schmertzler is a general and limited partner of, and trustee of certain family trusts holding interests in, Section Six Partners, L.P., and disclaims beneficial ownership of the shares held by Section Six Partners, L.P. except to the extent of any pecuniary interest therein.

In connection with the series four senior preferred stock financing, the outstanding convertible promissory notes converted into an aggregate of 502,919 shares of series four senior preferred stock and the outstanding warrants for series one preferred stock and series two preferred stock were automatically exercised for an aggregate of 2,095,515 shares of series five junior preferred stock.

Series one preferred stock financing and reclassification of outstanding preferred stock

In June and July 2012, we issued and sold an aggregate of 1,483,337 shares of our series one preferred stock, at a price per share of $20.00, for an aggregate purchase price of $29,666,740. In connection with the series one preferred stock financing, we also effected a reclassification of our previously outstanding preferred stock into an aggregate of 10,701,405 shares of series two preferred stock and 2,853,517 shares of series three preferred stock. Stockholders who participated in the series one preferred stock financing received series two preferred stock following the reclassification of our outstanding preferred stock. The following table sets forth the number of shares of our series one preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the number of shares of our series two preferred stock that we issued to our directors, executive officers and 5% stockholders and their affiliates upon the reclassification of our previously outstanding preferred stock.

Name	Shares of series one preferred stock purchased	Shares of series two preferred stock issued upon reclassification of outstanding preferred stock
Credit Suisse First Boston Equity Partners, L.P.(2)	328,604	2,706,450
HBM Healthcare Investments (Cayman) Ltd.(3)	274,232	1,529,875
Vulcan Ventures Incorporated and affiliates(4)	202,728	1,377,780
Celgene European Investment Company LLC(5)	152,749	1,435,000
Delphi Ventures and affiliates(6)	147,666	1,343,826
Section Six Partners, L.P.(7)	110,000	—

(1) In connection with the recapitalization and reverse stock split described above, the warrants described in the table above were automatically adjusted to be exercisable into shares of our series five preferred stock at the applicable conversion ratio.

(2) Consists of (i) 256,623 shares purchased and 2,090,351 shares issued upon reclassification held by Credit Suisse First Boston Equity Partners, L.P.; (ii) 71,733 shares purchased and 584,305 shares issued upon reclassification held by Credit Suisse First Boston Equity Partners (Bermuda), L.P.; (iii) 248 shares purchased and 2,019 shares issued upon reclassification held by Credit Suisse First Boston U.S. Executive Advisors, L.P. (iv) 1,875 shares issued upon reclassification held by Credit Suisse First Boston Finders & Screeners LP; and (v) 27,900 shares issued upon reclassification held by EMA Private Equity Fund 1999, LP. Mr. Schmertzler, a member of our board of directors, is a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P. and affiliates. Mr. Schmertzler disclaims beneficial ownership of the shares held by entities affiliated with Credit Suisse Credit Suisse First Boston Equity Partners, L.P. and affiliates except to the extent of any pecuniary interest therein.

(3) The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investments (Cayman) Ltd. is comprised of John Arnold, Richard Coles, Sophia Harris, Dr. Andreas Wicki and John Urquhart, none of whom has individual voting or investment power with respect to such shares, and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Mr. Bolte, a member of our board of directors, is an advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Mr. Bolte has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd., and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(4) Consists of (i) 812,500 shares issued upon reclassification held by Vulcan Ventures Incorporated and (ii) 202,728 shares purchased and 565,280 shares issued upon reclassification held by Vulcan Capital Venture Capital I LLC. Mr. Kranda, a member of our board of directors, is a consultant to Vulcan Incorporated Capital Venture Capital I LLC. Vulcan Ventures Incorporated and Vulcan Capital Venture Capital I LLC are each controlled by entities owned by Paul Allen. Mr. Kranda does not have voting or investment power over the shares held by Vulcan Ventures and its affiliates, and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(5) Dr. Zeldis, a member of our board of directors, is an employee of Celgene Corporation. Celgene European Investment Company LLC is a wholly owned subsidiary of Celgene Corporation. Dr. Zeldis disclaims beneficial ownership of the shares held by Celgene European Investment Company LLC except to the extent of any pecuniary interest therein.

(6) Consists of (i) 92,350 shares purchased and 656,367 shares issued upon reclassification held by Delphi Ventures V, L.P.; (ii) 1,001 shares purchased and 7,115 shares issued upon reclassification held by Delphi BioInvestments V, L.P.; (iii) 26,888 shares purchased and 336,804 shares issued upon reclassification held by Delphi Ventures VII, L.P.; (iv) 269 shares purchased and 3,368 shares issued upon reclassification held by Delphi BioInvestments VII, L.P.; (v) 26,895 shares purchased and 336,882 shares issued upon reclassification held by Delphi Ventures VIII, L.P.; and (vi) 263 shares purchased and 3,290 shares issued upon reclassification held by Delphi BioInvestments VIII, L.P.

(7) Mr. Schmertzler is a general and limited partner of, and trustee of certain family trusts holding interests in, Section Six Partners, L.P. Mr. Schmertzler disclaims beneficial ownership of the shares held by Section Six Partners, L.P. except to the extent of any pecuniary interest therein.

Placement agent fees

In connection with our series four preferred stock financing, we paid Credit Suisse Securities (USA) LLC a fee of $2,362,477 for its service as a placement agent for the transaction. Credit Suisse Securities (USA) LLC is an affiliate of Credit Suisse First Boston Equity Partners, L.P. and affiliated funds.

Registration rights

We are a party to an investors' rights agreement with the holders of our preferred stock, including some of our directors and 5% stockholders and their affiliates and entities affiliated with our directors. The investors' rights agreement provides these holders the right, following the completion of this offering, to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing. See "Description of capital stock—registration rights" for additional information regarding these registration rights.

Indemnification agreements

Our certificate of incorporation in effect upon the closing of this offering provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we have entered into indemnification agreements with our directors. See "Executive compensation—limitation of liability and indemnification" for additional information regarding these agreements.

Policies and procedures for related person transactions

Our board of directors has adopted written policies and procedures for the review of any transaction, arrangement or relationship in which PTC is a participant, the amount involved exceeds $120,000 and one of our executive officers, directors, director nominees or 5% stockholders, or their immediate family members, each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related person transaction," the related person must report the proposed related person transaction to our . The policy calls for the proposed related person transaction to be reviewed and, if deemed appropriate, approved by our audit committee. Whenever practicable, the reporting, review

and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chairman of the audit committee to review and, if deemed appropriate, approve proposed related person transactions that arise between committee meetings, subject to ratification by the committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

A related person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

- the related person's interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person's interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The audit committee may approve or ratify the transaction only if the committee determines that, under all of the circumstances, the transaction is in our best interests. The committee may impose any conditions on the related person transaction that it deems appropriate.

In addition to the transactions that are excluded by the instructions to the SEC's related person transaction disclosure rule, our board of directors has determined that the following transactions do not create a material direct or indirect interest on behalf of related persons and, therefore, are not related person transactions for purposes of this policy:

- interests arising solely from the related person's position as an executive officer of another entity (whether or not the person is also a director of such entity) that is a participant in the transaction, where (a) the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity, (b) the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction and do not receive any special benefits as a result of the transaction and (c) the amount involved in the transaction is less than the greater of $200,000 or 5% of the annual gross revenues of the company receiving payment under the transaction; and
- a transaction that is specifically contemplated by provisions of our charter or bylaws.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by the compensation committee in the manner specified in its charter.

We did not have a written policy regarding the review and approval of related person transactions prior to this offering. Nevertheless, with respect to such transactions, it was our policy for our board of directors to consider the nature of and business reason for such transactions, how the terms of such transactions compared to those which might be obtained from unaffiliated third parties and whether such transactions were otherwise fair to and in the best interests of, or not contrary to, our best interests. In addition, all related person transactions required prior approval, or later ratification, by our board of directors.

Principal stockholders

The following table sets forth information with respect to the beneficial ownership of our common stock as of March 15, 2013 by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled "Percentage of shares beneficially owned—before offering" is based on a total of 14,535,806 shares of our common stock outstanding as of March 15, 2012, including 13,795,956 shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering. The column entitled "Percentage of shares beneficially owned—after offering" is based on shares of our common stock to be outstanding after this offering, including the shares of our common stock that we are selling in this offering, but not including any additional shares issuable upon exercise of outstanding options or warrants.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options or warrants that are currently exercisable or exercisable within 60 days of March 15, 2013 are considered outstanding and beneficially owned by the person holding the options or warrants for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set

forth below, the address of the beneficial owner is c/o PTC Therapeutics Inc., 100 Corporate Court, South Plainfield, New Jersey 07080.

Name of beneficial owner	Shares beneficially owned	Percentage of shares beneficially owned	
		Before offering	After offering
Named executive officers and directors			
Stuart W. Peltz, Ph.D.(1)	197,552	1.4%	
Claudia Hirawat(2)	52,390	*	
Jay Barth, M.D.(3)	362	*	
Richard Aldrich(4)	583,333	4.0	
Axel Bolte(5)	–	*	
Allan Jacobson, Ph.D.(6)	39,852	*	
Adam Koppel, M.D., Ph.D.(7)	1,083,333	7.5	
Michael Kranda(8)	6,215	*	
Geoffrey McDonough, M.D.(9)	909	*	
Michael Schmertzler(10)	3,048,956	21.0	
David P. Southwell(11)	13,863	*	
Jerome B. Zeldis, Ph.D.(12)	1,071,212	7.4	
All executive officers and directors as a group (15 persons)	6,189,744	42.5	
5% stockholders			
Credit Suisse First Boston Equity Partners, L.P. and affiliates(13)	2,256,882	15.5	
HBM Healthcare Investments(Cayman) Ltd.(14)	1,841,495	12.7	
Vulcan Ventures Incorporated and affiliates(15)	1,368,398	9.4	
Brookside Capital Partners Fund, L.P.(16)	1,083,333	7.5	
Celgene European Investment Company LLC and affiliate(17)	1,071,212	7.4	
Delphi Ventures and affiliates(18)	955,766	6.6	
Section Six Partners, L.P.(19)	764,036	5.3	

* Less than one percent.

(1) Consists of (a) 8,749 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date and (b) 188,803 shares of common stock.

(2) Consists of (a) 2,333 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date and (b) 50,057 shares of common stock.

(3) Consists of 362 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date.

(4) Consists of 583,333 shares held by Longwood Fund LP. The managing members of Longwood Fund LP share voting and investment power with respect to the shares held by such entity. The managing members are Richard Aldrich, Michelle Dip and Christoph Westphal, each of whom disclaims beneficial ownership of the shares held by Longwood Fund LP except to the extent of any pecuniary interest therein.

(5) Mr. Bolte is an advisor to HBM Partners AG. HBM Partners AG acts as an investment advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Neither HBM Partners AG nor Mr. Bolte has voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd. and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. See also footnote 14.

(6) Consists of (a) 1,772 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date and (b) 38,080 shares of common stock.

(7) Consists of 1,083,333 shares held by Brookside Capital Partners Fund, L.P. Dr. Koppel disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. See also footnote 16.

(8) Consists of (a) 5,928 shares of common stock and (b) 287 shares issuable upon exercise of stock options exercisable within 60 days of March 15, 2013. See also footnote 15.

(9) Consists of 909 shares of common stock.

(10) Consists of (a) 2,256,882 shares held by Credit Suisse First Boston Equity Partners, L.P. and affiliates; (b) 764,036 shares held by Section Six Partners, L.P.; (c) 1,272 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date; and (d) 26,766 shares of common stock held by Mr. Schmertzler. Mr. Schmertzler disclaims beneficial ownership of the shares held by Credit Suisse First Boston Equity Partners, L.P. and its affiliates and by Section Six Partners, L.P., except in each case to the extent of his pecuniary interest therein. See also footnotes 13 and 19.

(11) Consists of (a) 625 shares of common stock underlying options that are exercisable as of March 15, 2013 or will become exercisable within 60 days after such date and (b) 13,238 shares of common stock.

(12) Consists of 1,071,212 shares held by Celgene European Investment Company LLC and affiliate. Dr. Zeldis disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. See also footnote 17.

(13) The address for Credit Suisse First Boston Equity Partners, L.P. and affiliates is Eleven Madison Avenue, 16th Floor, New York, NY 10010. Consists of (a) 1,759,609 shares held by Credit Suisse First Boston Equity Partners, L.P.; (b) 491,855 shares held by Credit Suisse First Boston Equity Partners (Bermuda), L.P.; (c) 3,487 shares held by EMA Private Equity Fund 1999, L.P.; (d) 1,697 shares held by Credit Suisse First Boston U.S. Executive Advisors, L.P.; and (e) 234 shares held by Credit Suisse First Boston Finders & Screeners, L.P. Mr. Schmertzler, a member of our board of directors, is a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P., who disclaims beneficial ownership of the shares held by entities affiliated with Credit Suisse Credit Suisse First Boston Equity Partners, L.P. except to the extent of any pecuniary interest therein.

(14) The address for HBM Healthcare Investments (Cayman) Ltd. is Centennial Towers, Suite 305, 2454 West Bay Road, Grand Cayman, Cayman Islands, B.V.I. The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investments (Cayman) Ltd. is comprised of John Arnold, Richard Coles, Sophia Harris, Dr. Andreas Wicki and John Urquhart, none of whom has individual voting or investment power with respect to such shares and each disclaims beneficial ownership of the shares held by HBM Healthcare Investments (Cayman) Ltd. except to the extent of any pecuniary interest therein. Mr. Bolte, a member of our board of directors, is an advisor to HBM Partners (Cayman) Ltd. HBM Partners (Cayman) Ltd. provides investment management services to HBM Healthcare Investments (Cayman) Ltd. Mr. Bolte has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd. and disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.

(15) The address for Vulcan Ventures Incorporated and affiliates is 505 Fifth Avenue, Suite 900, Seattle, WA 98104. Consists of (a) 797,102 shares held by Vulcan Capital Venture Capital I LLC; (b) 469,734 shares held by VCVC III LLC; and (c) 101,562 shares held by Vulcan Ventures Incorporated. Mr. Kranda, a member of our board of directors, is a consultant to Vulcan Capital Venture Capital I LLC. Vulcan Capital Venture Capital I LLC, VCVC III LLC, and Vulcan Ventures Incorporated are each controlled by entities owned by Paul Allen. Mr. Kranda does not have voting or investment power over the shares held by Vulcan Ventures Incorporated and its affiliates and disclaims beneficial ownership of such shares except to the extent of any beneficial ownership therein.

(16) The address for Brookside Capital Partners Fund, L.P. is John Hancock Tower, 200 Clarendon Street, Boston, MA 02116. Dr. Koppel, a member of our board of directors, is a Managing Director of Brookside Capital, LLC, the investment advisor to Brookside Capital Partners Fund, L.P. Dr. Koppel disclaims beneficial ownership of the shares held by Brookside Capital Partners Fund, L.P. except to the extent of any pecuniary interest therein.

(17) The address for Celgene European Investment Company LLC and affiliate is 86 Morris Avenue, Summit, NJ 07901. Consists of (a) 803,640 shares held by Celgene European Investment Company LLC and (b) 267,572 shares held by Celgene Corporation. Dr. Zeldis, a member of our board of directors, is an employee of Celgene Corporation. Celgene European Investment Company LLC is a wholly owned subsidiary of Celgene Corporation. Dr. Zeldis disclaims beneficial ownership of the shares held by Celgene European Investment Company LLC and Celgene Corporation except to the extent of any pecuniary interest therein.

(18) The address for Delphi Ventures and affiliates is 3000 Sand Hill Road, 1-135, Menlo Park, CA 94025. Consists of (a) 574,736 shares held by Delphi Ventures V, L.P.; (b) 6,225 shares held by Delphi BioInvestments V, L.P.; (c) 185,545 shares held by Delphi Ventures VII, L.P.; (d) 1,853 shares held by Delphi BioInvestments VII, L.P.; (e) 185,595 shares held by Delphi Ventures VII, L.P.; and (f) 1,812 shares held by Delphi BioInvestments VII, L.P.

(19) The address for Section Six Partners, L.P. is 1300 Valley Road, New Canaan, CT 06840. Mr. Schmertzler, a member of our board of directors, is a general and limited partner of, and trustee of certain family trusts holding interests in, Section Six Partners, L.P., who disclaims beneficial ownership of the shares held by Section Six Partners, L.P. except to the extent of any pecuniary interest therein.

Description of capital stock

General

The following description of our capital stock and provisions of our certificate of incorporation and bylaws are summaries and are qualified by reference to the certificate of incorporation and the bylaws that will be in effect upon the closing of this offering. We have filed copies of these documents with the SEC as exhibits to our registration statement of which this prospectus forms a part. The description of the capital stock reflects changes to our capital structure that will occur upon the closing of this offering.

Upon the closing of this offering, our authorized capital stock will consist of shares of our common stock, par value $0.001 per share, and shares of our preferred stock, par value $0.001 per share, all of which preferred stock will be undesignated.

As of March 15, 2013, we had issued and outstanding:

- 739,850 shares of our common stock held by 251 stockholders of record;
- 4,999,954 shares of our series four senior preferred stock that are convertible into 4,999,954 shares of our common stock; and
- 8,796,002 shares of our series five junior preferred stock that are convertible into 8,796,002 shares of our common stock;

As of March 15, 2013, we also had outstanding:

- options to purchase 46,638 shares of our common stock, at a weighted average exercise price of $ per share;
- warrants to purchase 644 shares of our common stock, at a weighted average exercise price of $390 per share; and
- warrants to purchase 16,368 shares of our series five junior preferred stock, at a weighted average exercise price of $128 per share.

Upon the closing of this offering:

- all of the outstanding shares of our preferred stock will automatically convert into an aggregate of 13,795,956 shares of our common stock;
- the warrants to purchase an aggregate of 644 shares of our common stock will remain outstanding and exercisable to purchase shares of our common stock, at a weighted average exercise price of $390 per share;
- the warrants to purchase 16,368 shares of our series five junior preferred stock at an exercise price of $128 per share will automatically become warrants to purchase 16,368 shares of our common stock, at an exercise price of $128 per share.

Common stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Each election of directors by our stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the

election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock.

In the event of our liquidation or dissolution, the holders of our common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any of our outstanding preferred stock. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

Preferred stock

Under the terms of our certificate of incorporation that will become effective upon the closing of this offering, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

Warrants

As of March 15, 2013, we had outstanding:

- warrants to purchase 644 shares of our common stock, at a weighted average exercise price of $390 per share; and
- warrants to purchase an aggregate of 16,368 shares of our series five junior preferred stock, at a weighted average exercise price of $128 per share.

Upon the closing of this offering, and after giving effect to the automatic conversion of our preferred stock into common stock, the warrants to purchase shares of our series five junior preferred stock will be exercisable, at the election of the holders, for an aggregate of shares of our common stock, at an exercise price of $ per share. These warrants provide for adjustments in the event of specified mergers, reorganizations, reclassifications, stock dividends, stock splits or other changes in our corporate structure. These warrants expire on January 28, 2020.

Options

As of March 15, 2013, options to purchase 46,638 shares of our common stock, at a weighted average exercise price of $ per share, were outstanding.

Delaware anti-takeover law and certain charter and by-law provisions

Delaware law

We are subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless either the interested stockholder attained such status with the approval of our board of directors, the business combination is approved by our board of directors and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of our outstanding voting stock in the transaction in which it became an interested stockholder. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person. The restrictions contained in Section 203 are not applicable to any of our existing stockholders that will own 15% or more of our outstanding voting stock upon the closing of this offering.

Staggered board; removal of directors

Our certificate of incorporation and our bylaws divide our board of directors into three classes with staggered three-year terms. In addition, our certificate of incorporation and our bylaws provide that directors may be removed only for cause and only by the affirmative vote of the holders of 75% of our shares of capital stock present in person or by proxy and entitled to vote. Under our certificate of incorporation and bylaws, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office. Furthermore, our certificate of incorporation provides that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder action; special meeting of stockholders; advance notice requirements for stockholder proposals and director nominations

Our certificate of incorporation and our bylaws provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our certificate of incorporation and our bylaws also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by our chairman of the board, our president or chief executive officer or our board of directors. In addition, our bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting, who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock, because even if it acquired a majority of our

outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

Super-majority voting

The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above.

Registration rights

We have entered into a second amended and restated investors' rights agreement, dated March 7, 2013, which we refer to as the investors' rights agreement, with the holders of our preferred stock. Upon the completion of this offering, holders of a total of shares of our common stock as of March 15, 2013, including shares issuable upon conversion of our preferred stock, will have the right to require us to register these shares under the Securities Act of 1933, as amended, or Securities Act, and to participate in future registrations of securities by us, under the circumstances described below. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. If not otherwise exercised, the rights described below will expire three years after the closing of this offering.

Demand registration rights

Beginning 180 days after the effective date of the registration statement of which this prospectus forms a part, subject to specified limitations set forth in the investors' rights agreement, at any time, the holders of 20% of the then-outstanding shares having rights under the investors' rights agreement, which we refer to as registrable shares, may at any time demand in writing that we register all or a portion of the registrable shares under the Securities Act if the total amount of registrable shares registered have an aggregate offering price of at least $10 million (net of selling expenses). We are not obligated to file a registration statement pursuant to this provision on more than two occasions, and we are not obligated to file a registration statement pursuant to this provision within 60 days before or 180 days after the effective date of any other registration statement that we may file or if we determine in good faith that it would be seriously detrimental to us or our stockholders.

Form S-3 registration rights

In addition, at any time after we become eligible to file a registration statement on Form S-3, subject to specified limitations set forth in the investors' rights agreement, the holders of registrable shares may demand in writing that we register on Form S-3 all or a portion of the registrable shares so long as the total amount of registrable shares being registered have an aggregate offering price of at least $5 million (net of selling expenses). We are not obligated to file a Form S-3 pursuant to this provision on more than four occasions, and we are not obligated to file a registration statement pursuant to this provision within 30 days before or 90 days after the effective date of any other registration statement that we may file or if we determine in good faith that it would be seriously detrimental to us or our stockholders.

Incidental registration rights

If, at any time after the closing of this offering, we propose to file a registration statement under the Securities Act, other than pursuant to the demand registration rights described above, the holders of registrable shares will be entitled to notice of the registration and, subject to specified exceptions, including market conditions, have the right to require us to register all or a portion of the registrable shares then held by them.

In the event that any registration in which the holders of registrable shares elect to participate pursuant to our investors' rights agreement is intended to be an underwritten public offering, we have agreed to enter into an underwriting agreement containing customary representation and warranties and covenants, including without limitation customary provisions with respect to indemnification of the underwriters of such offering. Holders of registrable securities must agree to any such underwriting agreement as a condition to participation in the offering.

Expenses

Pursuant to the investors' rights agreement, we are required to pay all registration expenses, including registration and filing fees, exchange listing fees, printing expenses and accounting fees and the fees and expenses of one counsel to represent the selling stockholders, in addition to any underwriting discounts and commissions, that are related to any demand or incidental registration described above. The registration rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

Transfer agent and registrar

The transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company, LLC.

NASDAQ global market

We have applied to have our common stock listed on The NASDAQ Global Market under the symbol "PTCT".

Shares eligible for future sale

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the anticipation of these sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of equity securities.

Upon the closing of this offering, we will have outstanding shares of our common stock, after giving effect to the issuance of shares of our common stock in this offering, assuming no exercise by the underwriters of their over-allotment option and no exercise of options or warrants outstanding as of .

Of the shares to be outstanding immediately after the closing of this offering, we expect that the shares to be sold in this offering will be freely tradable without restriction under the Securities Act unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act. The remaining shares of our common stock outstanding after this offering will be "restricted securities" under Rule 144, and we expect that substantially all of these restricted securities will be subject to the 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after this offering; and
- the average weekly trading volume of our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Upon expiration of the 180-day lock-up period described below, approximately shares of our common stock will be eligible for sale under Rule 144, including shares eligible for resale immediately

upon the closing of this offering as described above. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about us. Subject to the 180-day lock-up period described below, approximately shares of our common stock will be eligible for sale in accordance with Rule 701.

Lock-up agreements

We and each of our directors and executive officers and certain holders of our outstanding common stock, who collectively own shares of our common stock, based on shares outstanding as of , 2013, have agreed that, without the prior written consent of Credit Suisse, on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus, either directly or indirectly:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of any shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock, whether such transaction is to be settled by delivery of shares of our common stock or such other securities, in cash or otherwise, or publicly disclose an intention to do the same;

- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of our common stock, whether such transaction is to be settled by delivery of shares of our common stock or such other securities, in cash or otherwise; or

- make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for shares of our common stock, or with respect to the filing of any registration statement in connection therewith under the Securities Act.

The lock-up restrictions and specified exceptions are described in more detail under "Underwriting."

Registration rights

Upon the closing of this offering, the holders of shares of our common stock or their permitted transferees will be entitled to various rights with respect to the registration of these shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration, except for shares purchased by affiliates. See "Description of capital stock—registration rights" for additional information. Shares covered by a registration statement will be eligible for sale in the public market upon the expiration or release from the terms of lock-up agreements applicable to such shares.

Stock options

As of March 15, 2013, we had outstanding options to purchase 46,638 shares of our common stock, of which options to purchase 35,291 shares were vested. Following this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options and options and other awards issuable pursuant to the 2013 public company plan and our pre-IPO stock incentive plans. See "Executive compensation—stock option and other compensation plans" for additional information regarding these plans. Accordingly, shares of our common stock registered under the registration statements will be available for sale in the open market, subject to Rule 144 volume limitations applicable to affiliates, and subject to any vesting restrictions and lock-up agreements applicable to these shares.

Material federal U.S. tax considerations for non-U.S. holders of common stock

The following is a general discussion of material U.S. federal income and estate tax considerations relating to ownership and disposition of our common stock by a non-U.S. holder. For purposes of this discussion, the term "non-U.S. holder" means a beneficial owner of our common stock that is not, for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States or of any political subdivision of the United States;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust, if a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or if the trust has a valid election to be treated as a U.S. person under applicable U.S. Treasury Regulations.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any change could alter the tax consequences to non-U.S. holders described in this prospectus. In addition, the Internal Revenue Service, or the IRS, could challenge one or more of the tax consequences described in this prospectus.

We assume in this discussion that each non-U.S. holder holds shares of our common stock as a capital asset (generally, property held for investment). This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of U.S. state, local or non-U.S. taxes. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- financial institutions;
- brokers or dealers in securities;
- tax-exempt organizations;
- pension plans;
- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- insurance companies;
- controlled foreign corporations;
- passive foreign investment companies; and
- certain U.S. expatriates.

In addition, this discussion does not address the tax treatment of partnerships or persons who hold their common stock through partnerships or other entities which are pass-through entities for U.S. federal income tax purposes. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of the ownership and disposition of our common stock through a partnership or other pass-through entity, as applicable.

Prospective investors should consult their own tax advisors regarding the U.S. federal, state, local and non-U.S. income and other tax considerations of acquiring, holding and disposing of our common stock.

Dividends

If we pay distributions on our common stock, those distributions generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below under the heading "Gain on disposition of common stock." Any such distribution would also be subject to the discussion below under the section titled "Withholding and information reporting requirements—FATCA."

As discussed under "Dividend policy," we do not expect to pay cash dividends to holders of our common stock in the foreseeable future. In the event we do pay dividends, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States, and if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN (or successor form) and satisfy applicable certification and other requirements. Non-U.S holders are urged to consult their own tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim with the IRS.

Gain on disposition of common stock

A non-U.S. holder generally will not be subject to U.S. federal income tax on gain recognized on a disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States, and if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States; in these cases, the non-U.S. holder will be taxed on a net income basis at the regular graduated rates and in the manner applicable to U.S. persons, and if the non-U.S. holder is a corporation, an additional branch profits tax at a rate of 30%, or a lower rate as may be specified by an applicable income tax treaty, may also apply;

- the non-U.S. holder is a nonresident alien present in the United States for 183 days or more in the taxable year of the disposition and certain other requirements are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty) on the net gain derived from the disposition, which may be offset by U.S.-source capital losses of the non-U.S. holder, if any; or

- we are, or have been at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter), a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder held no more than 5% of our outstanding common stock, directly or indirectly, during the shorter of the five-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a "U.S. real property holding corporation" if the fair market value of its "U.S. real property interests" (as defined in the Code and applicable regulations) equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business.

Although there can be no assurance, we believe that we are not currently, and we do not anticipate becoming, a "U.S. real property holding corporation" for U.S. federal income tax purposes.

No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rule described above.

Information reporting and backup withholding

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate, currently 28%, with respect to dividends on our common stock. Generally, a holder will comply with such procedures if it provides a properly executed IRS Form W-8BEN (or other applicable Form W-8) or otherwise meets documentary evidence requirements for establishing that it is a non-U.S. holder, or otherwise establishes an exemption. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above under the heading "Dividends," will generally be exempt from U.S. backup withholding.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or

otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

Federal estate tax

Common stock owned or treated as owned by an individual who is a non-U.S. holder (as specially defined for U.S. federal estate tax purposes) at the time of death will be included in the individual's gross estate for U.S. federal estate tax purposes and, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

Withholding and information reporting requirements—FATCA

Recently enacted legislation, which is commonly referred to as "FATCA," will impose U.S. federal withholding tax of 30% on payments of dividends of, and gross proceeds from the sale or disposition of, our common stock if paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," the foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," the foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Although this legislation is effective with regards to amounts paid after December 31, 2012, under final regulations issued by the U.S. Department of Treasury on January 17, 2013, withholding under FATCA will only apply (1) to payments of dividends on our common stock made after December 31, 2013 and (2) to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2016. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits for such taxes.

Prospective investors should consult their own tax advisors regarding the possible impact of the FATCA rules on their investment in our common stock, and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of this 30% withholding tax under FATCA.

The preceding discussion of material U.S. federal tax considerations is for general information only. It is not tax advice. Prospective investors should consult their own tax advisors regarding the particular U.S. federal, state, local and non-U.S. tax consequences of purchasing, holding and disposing of our common stock, including the consequences of any proposed changes in applicable laws.

Underwriting

We are offering the shares of our common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC and Credit Suisse Securities (USA) LLC are acting as joint book running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the representatives. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of our common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	
Credit Suisse Securities (USA) LLC	
Cowen and Company, LLC	
Wedbush Securities Inc.	
Total	

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. After the initial public offering of the shares, the offering price and the selling concession may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to additional shares of our common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this over-allotment option. If any shares are purchased with this over-allotment option, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of our common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' over-allotment option.

	Without over-allotment exercise	With full over-allotment exercise
Per Share	$	$
Total	$	$

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or file with the Securities and Exchange Commission a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of our common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of our common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Credit Suisse Securities (USA) LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold hereunder and any shares of our common stock issued upon the exercise of options granted under our existing management incentive plans.

Our directors and executive officers, and certain of our significant stockholders have entered into lock up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 180 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC and Credit Suisse Securities (USA) LLC, (1) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers, managers and members in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant) or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock, in each case subject to certain exceptions, including (A) shares of common stock to be sold pursuant to the underwriting agreement, (B) transfers of shares of common stock or other securities as bona fide gifts, (C) transfers or dispositions of shares of common stock or other securities to any trust for the direct or indirect benefit of the director, officer or stockholder or the immediate family of such person in a transaction not involving a disposition for value, (D) transfers or dispositions of shares of common stock or other securities to any affiliate of the director, officer or stockholder or to any investment fund or other entity controlled or managed by such director, officer or stockholder; (E) transfers or dispositions of shares of common stock or other securities by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the director, officer or stockholder, and (F) distributions of shares of common stock or other securities to any of the stockholder's partners, members or stockholders. In the case of any transfer, disposition or distribution pursuant to clause (B), (C), (D), (E) or (F), each transferee, donee or distributee must execute and deliver to J.P.

Morgan Securities LLC and Credit Suisse Securities (USA) LLC a lock-up agreement. In addition, in the case of any transfer, disposition or distribution pursuant to clause (B), (C), (D) or (F), no filing by any party under the Exchange Act, or other public announcement reporting a reduction in the beneficial ownership of common stock held by the director, officer or stockholder, may be required or voluntarily made in connection with such transfer, disposition or distribution, other than a filing on a Form 5 made after the expiration of the 180-day period referred to above. In addition, notwithstanding the foregoing restrictions, the director, officer or stockholder may (i) exercise an option to purchase shares of common stock granted under any stock incentive plan or stock purchase plan, provided that the underlying shares of common stock continue to be subject to the restrictions on transfer set forth in the lock-up agreement, (ii) transfer the such stockholder's common stock or any security convertible into or exercisable or exchangeable for common stock to the Company pursuant to any contractual arrangement in effect on the date of the lock-up agreement that provides for the repurchase of such stockholder's common stock or such other securities by us or in connection with the termination of such stockholder's employment with us, (iii) establish a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of common stock, provided that such plan does not provide for any transfers of common stock, and no filing with the SEC or other public announcement shall be required or voluntarily made by the director, officer or stockholder or any other person in connection therewith, in each case during the 180-day restricted period pursuant to the lock-up agreement, and (iv) transfer or dispose of shares of common stock acquired in the offering, subject to certain restrictions with respect to company directed shares, or on the open market following the offering, provided that certain limitations on filings under the Exchange Act or other public announcements reporting a reduction in the beneficial ownership of common stock held by the director, officer or stockholder apply in connection with such transfer or disposition.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

We will apply to have our common stock approved for listing on The NASDAQ Global Market under the symbol "PTCT".

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of our common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of our common stock than they are required to purchase in this offering, and purchasing shares of our common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' over-allotment option referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their over-allotment option, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the over-allotment option. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act of 1933, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock,

including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The NASDAQ Global Market, in the over the counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account

of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

Selling restrictions

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State"), from and including the date on which the European Union Prospectus Directive (the "E.U. Prospectus Directive") was implemented in that Relevant Member State (the "Relevant Implementation Date") an offer of securities described in this prospectus may not be made to the public in that Relevant Member State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the competent authority in that Relevant Member State, all in accordance with the E.U. Prospectus Directive, except that, with effect from and including the Relevant Implementation Date, an offer of securities described in this prospectus may be made to the public in that Relevant Member State at any time:

- to any legal entity which is a qualified investor as defined under the E.U. Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the E.U. Prospectus Directive); or
- in any other circumstances falling within Article 3(2) of the E.U. Prospectus Directive, provided that no such offer of securities described in this prospectus shall result in a requirement for the publication by us of a prospectus pursuant to Article 3 of the E.U. Prospectus Directive.

For the purposes of this provision, the expression an "offer of securities to the public" in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the same may be varied in that Member State by any measure implementing the E.U. Prospectus Directive in that Member State. The expression "E.U. Prospectus Directive" means Directive 2003/71/EC (and any amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State) and includes any relevant implementing measure in each Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

Each of the underwriters has:

(1) only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000, or FSMA, received by it in connection with the issue or sale of the securities in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(2) complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the securities in, from or otherwise involving the United Kingdom.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

United Arab Emirates

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for the prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus you should consult an authorized financial advisor.

Legal matters

The validity of the shares of our common stock offered hereby is being passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP. Davis Polk & Wardwell LLP is acting as counsel for the underwriters in connection with this offering.

Experts

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements for each of the two years in the period ended December 31, 2012, as set forth in their report included in this prospectus. We have included our financial statements in this prospectus and elsewhere in this registration statement in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

Where you can find more information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document are not necessarily complete, and, in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference to such contract, agreement or document.

You may read and copy the registration statement of which this prospectus is a part at the SEC's public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, DC 20549. You can request copies of the registration statement by writing to the Securities and Exchange Commission and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC's public reference room. In addition, the SEC maintains an Internet website, which is located at http://www.sec.gov, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC's Internet website. Upon completion of this offering, we will be subject to the information reporting requirements of the Securities Exchange Act of 1934, and we will file reports, proxy statements and other information with the SEC.

PTC Therapeutics, Inc.
Index to financial statements

Report of independent registered accounting firm

The Board of Directors and Stockholders
PTC Therapeutics, Inc.

We have audited the accompanying balance sheets of PTC Therapeutics, Inc. (the Company) as of December 31, 2011 and 2012, and the related statements of operations, comprehensive income (loss), statements of convertible preferred stock and stockholders' deficit, and cash flows for each of the two years in the period ended December 31, 2012. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of PTC Therapeutics, Inc. at December 31, 2011 and 2012 and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

/s/ ERNST & YOUNG LLP

Metro Park, New Jersey
March 15, 2013

PTC Therapeutics, Inc.
Balance sheets

	December 31		Pro forma December 31 2012
	2011	2012	(Unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 28,431,410	$ 2,725,702	$
Prepaid expenses and other current assets	3,379,199	855,750	
Grant and collaboration receivables, net	1,244,128	1,013,813	
Total current assets	33,054,737	4,595,265	
Fixed assets, net	10,795,507	8,280,037	
Deposits and other assets	297,916	197,050	
	$ 44,148,160	$ 13,072,352	$
Liabilities convertible preferred stocks and stockholders' deficit			
Current liabilities:			
Accounts payable and accrued expenses	$ 13,049,454	$ 7,023,971	$
Current portion of long-term debt	7,139,975	4,444,171	
Deferred revenue	22,955,901	16,690,747	
Total current liabilities	43,145,330	28,158,889	
Deferred revenue, less current portion	16,448,656	741,667	
Long-term debt, less current portion	4,549,332	438,810	
Other long-term liabilities	4,229,617	2,549,719	
Total liabilities	68,372,935	31,889,085	
Commitments and contingencies *(Note 12)*			
Series One convertible preferred stock, designated 2,000,000 shares; issued and outstanding 1,483,337 shares at December 31, 2012	—	62,263,852	
Series Two convertible preferred stock, designated 13,750,000 shares; issued and outstanding 10,701,405 shares at December 31, 2012	—	18,182,129	
Series Three convertible preferred stock, designated 13,750,000 shares; issued and outstanding 2,853,517 shares at December 31, 2012	—	377,787	
Series A—G Convertible preferred stock:			
Preferred stock, $0.001 par value. Authorized 156,995,095 shares:			
Series A convertible preferred stock, designated 750,000 shares; issued and outstanding 750,000 shares at December 31, 2011 (liquidation preference of $750,000)	750,000	—	
Series B convertible preferred stock, designated 187,500 shares; issued and outstanding 187,500 shares at December 31, 2011 (liquidation preference of $375,000)	364,524	—	
Series C convertible preferred stock, designated 6,295,000 shares; issued and outstanding 6,000,000 shares at December 31, 2011 (liquidation preference of $15,000,000)	14,117,089	—	
Series D convertible preferred stock, designated 13,769,935 shares; issued and outstanding 13,095,769 shares at December 31, 2011 (liquidation preference of $42,561,249)	39,282,460	—	
Series E convertible preferred stock, designated 126,735,022 shares; issued and outstanding 125,740,607 shares at December 31, 2011 (liquidation preference of $49,999,998)	49,048,047	—	
Series E-2 convertible preferred stock, designated 3,670,138 shares; issued and outstanding 3,670,138 shares at December 31, 2011 (liquidation preference of $26,645,187)	26,509,451	—	
Series F convertible preferred stock, designated 675,000 shares; issued and outstanding 625,000 shares at December 31, 2011 (liquidation preference of $10,000,000)	10,000,000	—	
Series F-2 convertible preferred stock, designated 1,612,500 shares; issued and outstanding 1,515,503 shares at December 31, 2011 (liquidation preference of $24,248,048)	24,114,456	—	
Series G convertible preferred stock, designated 3,300,000 shares; issued and outstanding 3,143,750 shares at December 31, 2011 (liquidation preference of $50,300,000)	50,193,887	—	
Stockholders' deficit:			
Common stock, $0.001 par value. Authorized 216,666 shares; issued and outstanding 1,083 shares at December 31, 2011 and 4,526 shares at December 31, 2012	133	545	
Additional paid-in capital	12,007,007	177,583,672	
Accumulated deficit	(250,611,829)	(277,224,718)	
Total stockholders' deficit	(238,604,689)	(99,640,501)	
	$ 44,148,160	$ 13,072,352	$

See accompanying notes.

PTC Therapeutics, Inc.
Statements of operations

	Year ended December 31	
	2011	2012
Revenues:		
Collaboration revenue	$ 98,960,851	$ 28,779,078
Grant revenue	6,451,296	5,166,985
Total revenues and non-cash cancellation revenue	105,412,147	33,946,063
Operating expenses:		
Research and development	58,677,081	46,138,868
General and administrative	16,153,069	14,615,376
Total operating expenses	74,830,150	60,754,244
Income (loss) from operations	30,581,997	(26,808,181)
Interest expense, net	(2,444,417)	(1,209,577)
Other income, net	461,358	1,782,656
Income (loss) from operations before tax benefit	28,598,938	(26,235,102)
Tax benefit	2,305,576	—
Net income (loss)	30,904,514	(26,235,102)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	159,954,069
Less beneficial conversion charge	—	(377,787)
Net income attributable to common stockholders	$ 30,904,514	$ 133,341,180
Net income attributable to common stockholders per share:		
Basic	$ 23.95	$ 219.76
Diluted	$ 4.55	$ 42.50
Weighted-average shares outstanding:		
Basic	1,089	3,328
Diluted	5,729	17,205
Pro forma net income per share applicable to common stockholders—basic and diluted (unaudited)		$
Pro forma weighted-average number of shares outstanding (unaudited)		

See accompanying notes.

PTC Therapeutics, Inc.
Statements of comprehensive income (loss)

	Year ended December 31	
	2011	2012
Net income (loss)	$ 30,904,514	$ (26,235,102)
Other comprehensive income (loss):		
Unrealized loss on short-term investments	(3,606)	—
Comprehensive income (loss)	$ 30,900,908	$ (26,235,102)

See accompanying notes.

PTC Therapeutics, Inc.
Statements of convertible preferred stock and changes in stockholders' deficit
period from January 1, 2011 through December 31, 2012

	Series A—G convertible preferred stock		Series one—three convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' equity (deficit)
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance, January 1, 2011	154,728,267	$ 214,379,914	—	$ —	130,586	$ 131	$ 9,184,483	$ 3,606	$(281,516,343)	$(272,328,123)
Exercise of stock options	—	—	—	—	1,536	2	5,887	—	—	5,889
Share-based compensation expense	—	—	—	—	—	—	2,816,637	—	—	2,816,637
Net income	—	—	—	—	—	—	—	—	30,904,514	30,904,514
Unrealized loss on investments	—	—	—	—	—	—	—	(3,606)	—	(3,606)
Balance, December 31, 2011	154,728,267	214,379,914	—	—	132,122	133	12,007,007	—	(250,611,829)	(238,604,689)
Exercise of stock options	—	—	—	—	—	—	—	—	—	—
Conversion of Series E and E-2 convertible preferred stock to common stock	**(5,167,365)**	**(2,956,829)**	**—**	**—**	**413,223**	**412**	**2,956,417**	**—**	**—**	**2,956,829**
Issuance of Series One convertible preferred stock, exchange of Series A—G convertible preferred stock for Series Two and Series Three convertible preferred stock	**(149,560,902)**	**(211,423,085)**	**15,038,259**	**80,823,768**	**—**	**—**	**159,954,069**	**—**	**—**	**159,954,069**
Beneficial conversion charge	**—**	**—**	**—**	**—**	**—**	**—**	**377,787**	**—**	**(377,787)**	**—**
Share-based compensation expense	**—**	**—**	**—**	**—**	**—**	**—**	**2,288,392**	**—**	**—**	**2,288,392**
Net loss	**—**	**—**	**—**	**—**	**—**	**—**	**—**	**—**	**(26,235,102)**	**(26,235,102)**
Balance, December 31, 2012	**—**	**$ —**	**15,038,259**	**$80,823,768**	545,345	**$ 545**	**$177,583,672**	**$ —**	**$(277,224,718)**	**$ (99,640,501)**

See accompanying notes.

PTC Therapeutics, Inc.
Statements of cash flows
year ended December 31, 2011 and 2012

	Year ended December 31,	
	2011	2012
Cash flows from operating activities		
Net income (loss)	$ 30,904,514	$ (26,235,102)
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Depreciation	2,871,200	2,704,151
Change in valuation of warrant liability	(461,947)	(1,782,655)
Noncash interest expense	416,612	225,730
Share-based compensation expense	2,816,637	2,288,392
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,355,123)	2,452,430
Grant and collaboration receivables	2,052,550	230,315
Deposits and other assets	214,345	83,817
Accounts payable and accrued expenses	(3,159,711)	(6,025,483)
Other long-term liabilities	(67,151)	102,757
Deferred revenue	(54,998,713)	(21,972,143)
Net cash used in operating activities	(20,766,787)	(47,927,791)
Cash flows from investing activities		
Purchases of fixed assets	(165,116)	(188,681)
Purchases of investments	(2,019,163)	—
Maturities of investments	29,887,327	—
Net cash provided by (used in) investing activities	27,703,048	(188,681)
Cash flows from financing activities		
Payments on long-term debt	(7,185,610)	(6,943,988)
Net proceeds from sale of Series One preferred stock	—	29,354,752
Proceeds from issuance of common stock	5,889	—
Net cash (used in) provided by financing activities	(7,179,721)	22,410,764
Net decrease in cash and cash equivalents	(243,460)	(25,705,708)
Cash and cash equivalents, beginning of period	28,674,870	28,431,410
Cash and cash equivalents, end of period	$ 28,431,410	$ 2,725,702
Supplemental disclosure of cash information		
Cash paid for interest	$ 2,486,682	$ 1,211,764
Supplemental disclosures of noncash information related to investing and financing activities		
Change in unrealized loss on investments	$ (3,606)	$ —
Change in carry value of preferred securities resulting from the recapitalization	$ —	$ 159,954,069

See accompanying notes.

PTC Therapeutics, Inc.
Notes to financial statements
December 31, 2012

1. The Company

PTC Therapeutics, Inc. (the Company or PTC) was incorporated as a Delaware corporation on March 31, 1998. The Company is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed development of any drugs. The Company has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of December 31, 2012, the Company had an accumulated deficit of approximately $277.2 million. The Company has financed its operations to date primarily through private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and clinical trial support. The Company will need to obtain additional funding in connection with its ongoing operations. Additional financing may not be available to the Company. If the Company is unable to raise capital when needed or on attractive terms, the Company could be forced to delay, reduce or eliminate its research and development programs or any future commercialization efforts. As more fully described in Note 7, the Company completed a recapitalization of its outstanding convertible preferred stock in 2012 to enable the Company to raise additional capital.

Use of estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles (GAAP) requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

2. Summary of significant accounting policies

Unaudited pro forma presentation

The unaudited pro forma balance sheet information as of December 31, 2012 assumes the conversion of all outstanding shares of the Company's convertible preferred stock into s hares of common stock upon the closing of the Company's proposed initial public offering (IPO).

Unaudited pro forma net income per share is computed using the weighted-average number of common shares outstanding after giving effect to the pro forma effect of the conversion of all convertible preferred stock during the year ended December 31, 2012 into shares of the Company's common stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later.

Segment and geographic information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision making group, in deciding

how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment.

Accounting changes

Effective January 1, 2012, an update to an accounting standard was issued that requires that all nonowner changes in stockholders' equity be presented either in a single continuous statement of comprehensive income or in two separate but consecutive statements. This update was applied retrospectively. The Company adopted this pronouncement and elected to present a separate statement of comprehensive income. The updated standard does not change the items that must be reported in comprehensive income, how such items are measure, or when they must be reclassified to net income.

Cash equivalents

The Company considers all highly liquid investments with a maturity of 90 days or less at the time of purchase to be cash equivalents. Cash equivalents are carried at cost which approximates fair value due to their short-term nature.

Fixed assets

Fixed assets are stated at cost. Depreciation is computed starting when the asset is placed into service on a straight-line basis over the estimated useful life of the related asset as follows:

Leasehold improvements	Lesser of useful life or lease term
Computer equipment and software	3 years
Furniture, fixtures, and lab equipment	3 to 7 years

Grant and collaboration receivables

The Company records receivables in conjunction with grant and collaboration agreements as the revenue is invoiced. The Company will record an allowance for bad debt if receivables are anticipated to be uncollectible. There is no indication that any receivables are uncollectible as of December 31, 2011 and 2012. Write-offs of receivables have historically been insignificant.

Concentration of risks

The Company has no significant off-balance-sheet risk or credit risk concentrations. The Company maintains its cash and cash equivalents with various financial institutions. The Company maintains cash accounts that may at times exceed the federally insured limit; however, it has not experienced and does not anticipate experiencing any credit losses from maintaining cash accounts in excess of such limits.

The Company's revenues from its two largest collaboration partners and its largest grant as a percentage of total revenues were 85%, 8%, and 3%, respectively, for 2011 and were 67%, 11%, and 9%, respectively, for 2012.

Reverse stock split

As a result of the one-for-120 reverse stock split that was effected on March 7, 2013, each 120 shares of the Company's outstanding common stock were reclassified and combined into one share of common stock. All references to common stock have been restated to reflect the reverse stock split on a retroactive basis.

Deferred rent

The Company has an operating lease for office space. Rent expense is recorded on a straight-line basis over the initial lease term. The difference between the actual cash paid and the straight-line rent expense is recorded as deferred rent. Leasehold improvements made related to this lease, subsequent to its inception, are amortized over the remaining lease term.

Revenue recognition

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

The Company's revenue is generated primarily through collaborative research and development and licensing agreements and grants.

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

For existing collaborations entered into prior to the adoption in 2011 of the revised multiple element revenue recognition guidance described below, the Company recognized revenue consistent with the approach established at the inception of each arrangement. For these existing collaborations, where the Company has continuing involvement, the Company recorded nonrefundable, upfront fees as deferred revenue and recognizes revenue on a straight-line basis as collaboration revenue over the expected performance period.

For new collaborations or for material modifications made to existing collaborations, in 2011 and thereafter, the Company adopted the updated multiple element revenue recognition guidance. Under this new guidance, all non-contingent arrangement consideration is allocated to the identified units of accounting based on their relative selling price at inception of the collaboration arrangement. The Company derives the selling price using a combination of internal subjective and available external objective information, such as comparable transactions. The Company recognizes revenue commensurate with delivery, such as in the case with delivery of a license, or ratably over the course of a service period, as appropriate, such as in the case of ongoing research and development activities.

The Company evaluates all contingent consideration earned, such as a milestone payment, using the criteria as provided by the Financial Accounting Standards Board (FASB), guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, the Company evaluates if milestone payments are substantive. The criteria requires that (1) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as substantive milestones and will be recognized as revenue in the period that the milestone is achieved. The Company recognizes royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

Research and development costs

Research and development expenses include the clinical development costs associated with the Company's product development programs and research and development costs associated with the Company's discovery programs. These expenses include internal research and development costs and the costs of research and development conducted on behalf of the Company by third parties, including sponsored university-based research agreements and clinical study vendors. All research and development costs are expensed as incurred. Costs incurred in obtaining technology licenses are charged immediately to research and development expense if the technology licensed has not reached technological feasibility and has no alternative future uses.

Nonrefundable advance payments made for goods and services that will be used in future research and development activities are deferred if the contracted party has not yet performed the related activities. The amount deferred is then recognized as expense when the research and development activities are performed. The Company has deferred research and development advance payments of approximately $245,000 and $228,000 as of December 31, 2011 and 2012, respectively.

Fair value of financial instruments

The Company follows the fair value measurement rules, which provides guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules establishes a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.

- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).

- Level 3—Inputs are unobservable and reflect the Company's assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents and investments are reflected in the accompanying financial statements at fair value. The carrying amount of grant and collaboration receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments.

Beneficial conversion

When the Company issues a debt or an equity security that is convertible into common stock at a discount from the fair value of the common stock at the date the debt or equity security counterparty is legally committed to purchase such a security (Commitment Date), a beneficial conversion charge is measured and recorded on the Commitment Date for the difference between the fair value of the Company's common stock and the effective conversion price of the convertible debt or equity security. If the intrinsic value of the beneficial conversion feature is greater than the proceeds allocated to the convertible debt or equity

security, the amount of the discount assigned to the beneficial conversion feature is limited to the amount of the proceeds allocated to the convertible debt or equity security.

The amount allocated to the beneficial conversion feature is presented as a discount or reduction to the related debt security or as an immediate charge to earnings available to common shareholders for convertible preferred stock instruments that are convertible by the shareholders at any time. In connection with the Company's recapitalization of its outstanding convertible preferred stock in 2012, the Company recorded a beneficial conversion charge representing the difference between the effective conversion price and the fair value of the Company's common stock as of the Commitment Date. Because the intrinsic value was in excess of the proceeds allocated to the Company's new Series Three convertible preferred stock; the beneficial conversion charge was limited to the allocated proceeds of approximately $377,000.

Warrant liability

Warrants to purchase the Company's common stock with nonstandard antidilution provisions and preferred stock that include a put feature, regardless of the probability or likelihood that may conditionally obligate the issuer to ultimately transfer assets, are classified as liabilities and are recorded at their estimated fair value at each reporting period. Any change in fair value of these warrants is recorded as gain/(loss) on warrant valuation each reporting period in Other income on the Company's statement of operations.

Impairment of long-lived assets

The Company monitors its long-lived assets for indicators of impairment. If such indicators are present, the Company assesses the recoverability of affected assets by determining whether the carrying value of such assets is less than the sum of the undiscounted future cash flows of the assets. If such assets are found not to be recoverable, the Company measures the amount of such impairment by comparing the carrying value of the assets to the fair value of the assets, with the fair value generally determined based on the present value of the expected future cash flows associated with the assets. Although current and historical negative cash flows are indicators of impairment, management believes the future cash flows to be received from the long-lived assets and the potential success of the Company's research programs will exceed the assets' carrying value, and accordingly, the Company believes that no impairment of long-lived assets exists as of December 31, 2012.

Share-based compensation

The Company measures the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the entire award.

The fair value of options is calculated using the Black-Scholes option pricing model to determine the fair value of stock options on the date of grant based on key assumptions such as stock price, expected volatility and expected term. The Company's estimates of these assumptions are primarily based on third-party valuations, historical data, peer company data and judgment regarding future trends and factors.

The Company utilized various valuation methodologies in accordance with the framework of the 2004 American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its stock. The methodologies included an option pricing method to estimate the Company's underlying equity value, and a methodology that determined an estimated value under an initial public offering (IPO) scenario and a sale scenario based upon an assessment of the probability of occurrence of each scenario. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates include assumptions regarding future performance, including the completion of clinical trials and the time

to complete an IPO or sale of the Company. As with any valuation, significant changes to the key assumptions used in the valuations could result in different fair values of common stock at each valuation date.

Income taxes

Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and net operating loss and credit carryforwards. Deferred tax assets and liabilities are measured at rates expected to apply to taxable income in the years in which those temporary differences and carryforwards are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date. A valuation allowance is recorded when it is not more likely than not that all or a portion of the net deferred tax assets will be realized.

Net income per share

Basic net income per share is calculated by dividing the net income attributable to common stockholders by the weighted average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net income per share is calculated by dividing the net income attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method and the if-converted method. Dilutive common stock equivalents are comprised of convertible preferred stock and options outstanding under the Company's stock option plans.

3. Fair value of financial instruments and investments

Fair value of certain investments is based upon market prices using quoted prices in active markets for identical assets quoted on the last day of the year. In establishing the estimated fair value of the remaining investments, the Company used the fair value as determined by its investment advisors using observable inputs other than quoted prices.

The Company reviews its investments on a periodic basis for other-than-temporary impairments. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may have a significant adverse effect on the fair value of the investment. As of December 31, 2011, the Company deemed its unrealized losses not to be other-than-temporary.

The following represents the fair value using the hierarchy described in Note 2 for the Company's financial assets that are required to be measured at fair value on a recurring basis as of December 31, 2011 and 2012:

	December 31, 2011			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Warrant liability	$1,878,316	$ —	$ —	$ 1,878,316

	December 31, 2012			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Warrant liability	$ 95,661	$ —	$ —	$ 95,661

Level 3 valuation

The warrant liability is classified in Other long-term liabilities on the Company's balance sheet. The warrant liability is marked-to-market each reporting period with the change in fair value recorded as a gain or loss within Other income on the Company's statement of operations until the warrants are exercised, expire or other facts and circumstances lead the warrant liability to be reclassified as an equity instrument. The fair value of the warrant liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuation for warrant liability for the years ended December 31, 2011 and 2012:

	Level 3 assets
Beginning balance January 1, 2011	$ 2,340,263
Change in fair value of warrant liability	(461,947)
Ending balance as of December 31, 2011	1,878,316
Change in fair value of warrant liability	(1,782,655)
Ending balance as of December 31, 2012	$ 95,661

Fair value of the warrant liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the preferred stock value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2011 and 2012 include (i) volatility, (ii) risk free interest rate, (iii) strike price, (iv) fair value of preferred shares, and (v) expected life. The fair value of the preferred shares declined significantly due to the recapitalization of the Company's outstanding convertible preferred stock in June 2012 as described in Note 7.

4. Fixed assets

Fixed assets, net were as follows at December 31, 2011 and 2012:

	December 31,	
	2011	2012
Leasehold improvements	$ 12,473,836	$ 12,473,836
Computer equipment and software	2,082,638	2,118,713
Furniture, fixtures, and lab equipment	13,781,118	13,969,758
Assets not yet placed in service	40,186	4,152
	28,377,778	28,566,459
Less accumulated depreciation and amortization	(17,582,271)	(20,286,422)
	$ 10,795,507	$ 8,280,037

Depreciation expense was approximately $2,871,000 and $2,704,000 for the years ended December 31, 2011 and 2012, respectively.

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at December 31, 2011 and 2012 consist of the following:

	December 31,	
	2011	2012
Employee compensation, benefits, and related accruals	$ 3,492,170	$ 3,096,475
Consulting and contracted research	6,749,272	2,515,678
Professional fees	492,811	559,228
Accounts payable	1,878,109	621,591
Other	437,092	230,999
	$ 13,049,454	$ 7,023,971

6. Long-term debt

In May 2009, the Company entered into a capital lease for a laboratory instrument. This lease carries an implied interest rate of 8.2% and is payable in fixed monthly installments. As of December 31, 2011 and 2012, the Company had approximately $314,000 and $187,000 of remaining principal, respectively, which approximates the fair value.

In September 2009, the Company entered into a $25,000,000 secured debt facility with a syndicate of two lenders. In conjunction with entering into the debt facility, the Company issued warrants to purchase 62,500 shares of Series F-2 convertible preferred stock at an exercise price of $16.00 per share to the lenders. The warrants became exercisable in proportion to the amount of the facility borrowed. The fair value of the warrants was reflected as a discount to debt, and this discount is accreted to interest expense over the term of the debt facility.

The Company borrowed $12,500,000 under the facility in September 2009 and an additional $10,000,000 under the facility in December 2010 and issued the lenders promissory notes. The notes are secured by substantially all of the Company's assets except for intellectual property. The notes carry a fixed interest

rate of 13.65% and required interest-only payments for the first five months, with principal repayment beginning in month six and continuing for 30 months. As of December 31, 2011 warrants to acquire 56,250 shares of Series F-2 were exercisable. In connection with the recapitalization of the Company's outstanding convertible preferred stock in 2012, these warrants were amended to be warrants to purchase Series Two convertible preferred stock. As of December 31, 2011 and 2012, the outstanding balance on the notes was $11,569,000 and $4,752,000, respectively. The carrying amount of the notes approximates their fair values based on the short maturity of the notes.

The debt facility has certain representations, warranties and affirmative covenants, as well as certain negative covenants restricting our activities, including limitations on dispositions, mergers or acquisitions, incurring indebtedness or liens, paying dividends or making investments and certain other business transactions. There are no financial covenants associated with the debt facility.

The debt facility contains certain events of default. The obligations under the debt facility and the other loan documents may at the lenders' option be accelerated upon the occurrence of certain events of default, and are automatically accelerated upon certain bankruptcy and insolvency related events of default. As of December 31, 2012, there were no events of default under the debt facility.

As of December 31, 2012, aggregate debt maturities are as follows:

2013	$ 4,499,442
2014	439,487
Total debt at maturity value	4,938,929
Less unamortized discount	(55,948)
Total carrying value of debt	$ 4,882,981

7. Capital structure

Convertible preferred stock prior to 2012 recapitalization

As of December 31, 2011, the Company had authorized for issuance up to 156,995,095 shares of preferred stock, $0.001 par value. The authorized shares as of December 31, 2011 were designated as follows: 750,000 shares of Series A convertible preferred stock (Series A), 187,500 shares of Series B convertible preferred stock (Series B), 6,295,000 shares of Series C convertible preferred stock (Series C), 13,769,935 shares of Series D convertible preferred stock (Series D), 126,735,022 shares of Series E convertible preferred stock (Series E), 3,670,138 shares of Series E-2 convertible preferred stock (Series E-2), 675,000 shares of Series F convertible preferred stock (Series F), 1,612,500 shares of Series F-2 convertible preferred stock (Series F-2) and 3,300,000 shares of Series G convertible preferred stock (Series G).

The rights and preferences of the shares of Series A, Series B, Series C, Series D, Series E, Series E-2, Series F, Series F-2 and Series G were as follows:

Conversion—Each share of Series A, Series B, Series C, Series D, Series E, Series E-2, Series F, Series F-2 and Series G was convertible at any time at the option of the holder into such number of shares of common stock as determined by applying a conversion factor to the outstanding shares of approximately 0.0833, 0.1333, 0.1389, 0.1548, 0.0548, 1.0000, 1.0000, 1.0000 and 1.0000 for the Series A, Series B, Series C, Series D, Series E, Series E-2, Series F, Series F-2, and Series G respectively. These conversion factors were calculated based on the then-applicable conversion price with respect to each respective series of preferred stock. These conversion factors were subject to adjustment in the event the Company

issued additional equity securities at prices below the then-applicable conversion price, or if the Company engaged in specified changes to its capitalization, such as stock splits or stock dividends. The conversion of each series of preferred stock would be automatic upon the closing of a qualified initial public offering or any other public offering upon the written election of the Company and holders of both (1) at least two thirds of the outstanding preferred shares on an as-converted to common stock basis and (2) at least two thirds of the Series F, F-2 and G shares voting together as a single class on an as-converted to common stock basis.

Voting—Each preferred shareholder was entitled to the number of votes per share as if the preferred shares were converted to common stock. Additionally, the holders of the preferred stock, voting as a single class, were entitled to elect six members of the Board of Directors.

Liquidation—Upon the liquidation, dissolution, reorganization or winding-up of the Company, holders of preferred stock were entitled to receive, before any distribution or payment on the common stock, an amount equal to $1.00 per share for Series A, $2.00 per share for Series B, $2.50 per share for Series C, $3.25 per share for Series D, $0.397644 per share for Series E, $7.26 per share for Series E-2, $16.00 per share for Series F, $16.00 per share for Series F-2, and $16.00 per share for Series G, plus all declared, but unpaid, dividends. As of December 31, 2011, the aggregate liquidation preference was $750,000, $375,000, $15,000,000, $42,561,249, $49,999,998, $26,645,187, $10,000,000, $24,248,048, and $50,300,000 for the Series A, Series B, Series C, Series D, Series E, Series E-2, Series F, Series F-2, and Series G, respectively. In cases where the liquidation preference applied, if there were insufficient funds to pay the full preference value to all holders, then, as a group, the holders of the Series E, Series E-2, Series F, Series F-2, and Series G would have been paid together first, ratably, in proportion to their respective liquidation preferences. To the extent there were excess assets to distribute, the holders of the Series D would have been paid second. Finally, as a group, the holders of the Series A, Series B, and Series C would have been paid last, ratably, in proportion to their respective liquidation preferences. Dividends were payable only if and when declared. The Company has not declared any dividends through December 31, 2012.

Recapitalization

In July 2012, the Company completed a recapitalization pursuant to which all outstanding shares of Series A, B, C, D, E, E-2, F, F-2, and G convertible preferred stock (Prior Series Preferred) were exchanged into Series Three convertible preferred stock (Series Three). Warrants to acquire Prior Series Preferred became warrants to acquire Series Two convertible preferred stock (Series Two). In addition, those investors that elected to participate in the sale of Series One convertible preferred stock (Series One) were entitled to exchange their Series Three shares for Series Two shares.

In connection with the recapitalization, the Company sold 1,483,337 shares of Series One for aggregate gross proceeds of approximately $29.7 million.

The Company accounted for the recapitalization as an extinguishment of its Prior Series Preferred and recorded the Series One, Series Two and Series Three shares at their fair value as of the recapitalization date. In accordance with authoritative accounting guidance, the Company recorded a gain attributable to the common stockholders on the extinguishment of the Prior Series Preferred. The gain of approximately $160 million represents the excess of the carrying amount of Prior Series Preferred stock immediately prior to the recapitalization over the fair value of the Series One, Two and Three stock issued in connection with the recapitalization.

The rights and preferences of the shares of Series One, Two and Three are as follows:

Dividends—The holders of Series One and Series Two, in preference to the holders of common stock, are entitled to noncumulative dividends when and if declared by the Board of Directors. The holders of Series Three are not entitled to dividends. The Company has not declared any dividends through December 31, 2012.

Liquidation—Upon the liquidation, dissolution, reorganization or winding-up of the Company, the holders of Series One will be entitled to receive, before any distribution or payment is made to any other class of security, an amount equal to two times the original issuance price, plus all declared, but unpaid, dividends. To the extent there are excess assets to distribute, the holders of Series Two will be entitled to receive 76.47% of such excess assets, and the holders of Series One will be entitled to receive 23.53% of such excess assets, until the holders of Series Two receive an amount equal to one times the stated liquidation preference amount for the Series Two, plus all declared, but unpaid, dividends. In the event there are remaining assets after Series Two distributions, the holders of Series Three are entitled to receive 8.82% of such remaining assets, and the holders of Series One and Series Two will be entitled to receive 23.53% and 67.65%, respectively, of such remaining assets, until the holders of Series Three receive an amount equal to one times the stated liquidation preference amount for the Series Three, plus all declared, but unpaid, dividends. To the extent there are remaining assets to distribute, the holders of Series One, Series Two, and Series Three will be entitled to receive 20%, 55%, and 25% of such remaining assets, respectively.

Voting—Each holder of Series One is entitled to cast the number of votes equal to five times the number of common shares into which such holder's shares of Series One would convert. Except as required by law, holders of Series Two and Series Three have limited voting rights. Additionally, the holders of Series One, voting as a single class, are entitled to elect twelve members of the Board of Directors.

Conversion—Each share of Series One is convertible at any time at the option of the holder into two shares of common stock. Each share of Series Two and Series Three is convertible at any time at the option of the holder into one share of common stock. These conversion ratios are subject to adjustment for certain dilutive events, including certain types of stock splits or stock dividends or future recapitalizations.

Warrants

All of the Company's outstanding warrants are classified as liabilities as of December 31, 2011 and 2012 because they contain either non-standard antidilution provisions or they are exercisable into preferred shares that include a put feature.

The following is a summary of the Company's outstanding warrants as of December 31, 2011:

	Warrant shares	Exercise price	Expiration
Series E convertible preferred stock	54,465	$ 7.26	2014
Series F convertible preferred stock	50,000	$ 16.00	2017
Series F-2 convertible preferred stock	56,250	$ 16.00	2019 and 2020
Common stock	644	$ 390	2013 and 2014

In connection with the recapitalization, all of the Series E, F, and F-2 outstanding warrants became warrants to purchase Series Two.

The following is a summary of the Company's outstanding warrants as of December 31, 2012:

	Warrant shares	Exercise price	Expiration
Series 2 convertible preferred stock	24,712	$ 16.00	2014
Series 2 convertible preferred stock	50,000	$ 16.00	2017
Series 2 convertible preferred stock	56,250	$ 16.00	2019 and 2020
Common stock	644	$ 390	2013 and 2014

8. Earnings per share

Basic earnings per share is computed by dividing net income available to common stockholders by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net income available to common stockholders by the weighted-average number of common shares plus the effect of dilutive potential common shares outstanding during the period.

The Prior Series Preferred outstanding in 2011 and 2012 (through the date of the recapitalization) as well as Series One and Series Two outstanding during 2012 (subsequent to the recapitalization) participate in earnings of the Company through dividend rights. Accordingly, the Company measures earnings per share based upon the two-class method. Net income attributable to common stockholders excludes $30,878,445 and $132,609,918 for the years ended 2011 and 2012, respectively, for net income attributable to participating securities.

The diluted earnings per share for the years ended December 31, 2011 and 2012 exclude the impact of approximately 1.3 million and 0.6 million common stock equivalents, respectively, since the effect of including these securities would be anti-dilutive.

The following table sets forth the computation of basic and diluted earnings per share for common stockholders:

Net income per share

	Year ended December 31	
	2011	2012
Numerator		
Net income (loss)	$ 30,904,514	$ (26,235,102)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	159,954,069
Less beneficial conversion charge	—	(377,787)
Less net income attributable to participating preferred stock	(30,878,445)	(132,609,918)
Net income attributable to common stockholders	$ 26,069	$ 731,262
Denominator		
Denominator for basic earnings per share	1,089	3,328
Effect of dilutive securities:		
Employee stock options	4,640	—
Series 3 convertible preferred stock	—	13,877
Denominator for diluted earnings per share	5,729	17,205
Net income per share:		
Basic	23.95	219.76
Diluted	4.55	42.50

9. Stock option plan

In 2009, the Company's shareholders approved the 2009 Equity and Long-Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards, subject to certain adjustments and annual increases. As of December 31, 2012, awards for 14,271 shares of common stock are available for issuance.

The Board of Directors has the authority to select the individuals to whom options are granted and determine the terms of each option, including (i) the number of shares of common stock subject to the option; (ii) the date on which the option becomes exercisable; (iii) the option exercise price, which, in the case of incentive stock options, must be at least 100% (110% in the case of incentive stock options granted to a stockholder owning in excess of 10% of the Company's stock) of the fair market value of the common stock as of the date of grant; and (iv) the duration of the option (which, in the case of incentive stock options, may not exceed ten years). Options typically vest over a three- or four-year period.

A summary of stock option activity is as follows:

	Number of options	Exercise price	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value
Outstanding at December 31, 2010	39,557	$226.80–$1,149.60	$ 547.20		
Granted	8,123	$490.80	$ 490.80		
Exercised	(11)	$451.20–$508.80	$ 459.60		
Forfeited	(875)	$226.80–$1,149.60	$ 757.20		
Outstanding at December 31, 2011	46,794	$226.80–$1,149.60	$ 532.80	5.74 years	
Granted	5,715	$218.40	$ 218.40		
Exercised	—				
Forfeited	(10,115)	$218.40–$1,149.60	$ 604.80		
Outstanding at December 31, 2012	42,394	$218.40–$1,149.60	$ 474.00	5.02 years	
Exercisable at December 31, 2012	33,414	$218.40–$1,149.60	$ 482.40	4.14 years	
Exercisable and expected to vest at December 31, 2012	42,184	$218.40–$1,149.60	$ 476.40	4.97 years	

The fair value of grants made in the years ended December 31, 2011 and 2012 was contemporaneously estimated on the date of grant using the following assumptions:

	2011	2012
Risk-free interest rate	2.40%	1.135%
Expected volatility	87%	87%
Expected term	6.00–6.25 years	6.00–6.25 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the years ended December 31, 2011 and 2012 was $364.80 and $160.80, respectively.

The Company uses the "simplified method" to determine the expected term of options. Under this method, the expected term represents the average of the vesting period and the contractual term. The expected

volatility of share options was estimated based on a historical volatility analysis of peers that were similar to the Company with respect to industry, stage of life cycle, size, and financial leverage. The risk-free rate of the option is based on U.S. Government Securities Treasury Constant Maturities yields at the date of grant for a term similar to the expected term of the option.

The Company recognized approximately $2,817,000 and $2,288,000 of share-based compensation expense, during the years ended December 31, 2011 and 2012, respectively. The Company utilizes newly issued shares to satisfy stock option exercises.

As of December 31, 2011 and 2012, there was approximately $4,005,000 and $2,213,000, respectively of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 1998 and 2009 Plan. This cost is expected to be recognized as compensation expense over the weighted average remaining service period of approximately 2.14 years.

10. Collaborations and grants

The Company has ongoing collaborations with the Spinal Muscular Atrophy Foundation (SMA Foundation) and F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc. (collectively, Roche) and early stage discovery arrangements with other institutions. During 2011, the Genzyme collaboration was modified and later terminated. The following are the key terms to the Company's (i) terminated collaboration with Genzyme, (ii) ongoing collaborations and (iii) early stage discovery and development arrangements.

Terminated collaboration

Genzyme

In July 2008, Genzyme Corporation (now a Sanofi company) and the Company entered into an exclusive global collaboration to develop and commercialize ataluren, the Company's novel oral therapy in late-stage development for the treatment of genetic disorders due to nonsense mutations. Under the terms of this agreement, the Company granted Genzyme rights to commercialize ataluren in all countries except the United States and Canada, which rights the Company retained. Genzyme made a nonrefundable upfront payment to the Company of $100,000,000 in July 2008, which was being recognized over the Company's estimated period of performance under the arrangement.

In August 2011, the Company and Genzyme announced a restructuring of the agreement. Under the terms of the restructuring, the Company regained worldwide rights to ataluren and Genzyme made an additional payment to the Company in exchange for an option to commercialize ataluren in indications other than nonsense mutation Duchenne muscular dystrophy (nmDMD) outside the United States and Canada. On March 27, 2012, the Company received notification that Genzyme declined to exercise the option, at which time the option expired. As a result, the collaboration was terminated.

The Company evaluated the August 2011 restructuring of the Genzyme collaboration agreement and determined it to be a material modification to the original agreement for financial reporting purposes pursuant to the revised multiple element revenue recognition guidance. The Company reevaluated the collaboration arrangement under this revised guidance and recorded a one-time adjustment to its deferred revenue balance to reflect the value of the remaining performance obligations under the restructured agreement (represented by the best estimate of selling price). The effect of this reevaluation was to recognize approximately $79 million of existing deferred revenue as of the restructuring date. For the years ended December 31, 2011 and 2012, the Company recognized approximately $90,012,000 and $3,756,000, respectively, in collaboration revenue from Genzyme.

Current collaboration

Roche and SMA Foundation

In November 2011, the Company and the SMA Foundation entered into a licensing and collaboration agreement with Roche for a spinal muscular atrophy program. Under the terms of the agreement, Roche acquired an exclusive worldwide license to the Company's spinal muscular atrophy program, which includes three compounds currently in preclinical development, as well as potential back-up compounds. The Company received a nonrefundable upfront cash payment of $30,000,000 and Roche agreed to provide funding for research activities performed on its behalf.

The Company applied the revised multiple element revenue recognition guidance in evaluating the accounting treatment of this collaboration agreement. The Company identified two possible significant deliverables in the collaboration agreement, the license and the research activities. The Company evaluated whether these significant deliverables have stand alone value and determined that the license does not have standalone value without the ongoing research and development services given the unique nature of the technology. As such, both of these elements were combined as a single unit for accounting purposes. As a result, the Company deferred the $30,000,000 upfront payment which is being recognized over the estimated performance period. For the year ended December 31, 2011 and 2012, the Company recognized approximately $2,072,000 and $18,405,000, respectively, in collaboration revenue. The balance of the remaining deferred upfront payment was approximately $13,417,000 at December 31, 2012.

Under the agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135 million in research and development event milestones, up to $325 million in sales milestones upon achievement of sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

The Company considers that each of the potential milestone events under the agreement would be substantive because the applicable criteria of its revenue recognition policy (see Note 2) would be satisfied.

Early stage collaboration and discovery agreements

The Company has arrangements with several organizations pursuant to which the Company uses its discovery technologies to help identify potential drug candidates. The Company does not take ownership of the potential compounds, but rather provides research services to the collaborator using its specialized technology platform.

Generally, these arrangements are structured such that the collaborator and the Company work together to jointly select targets from which to apply its discovery technologies. The research period for the Company to apply its technology is generally three to four years. The Company will typically receive a nonrefundable, upfront cash payment and the collaborator agrees to provide funding for research activities performed on its behalf.

For those arrangements entered into or significantly modified after January 1, 2011, the Company applies the revised multiple element revenue recognition guidance in evaluating the accounting treatment for these arrangements. Generally, the two significant deliverables in these arrangements are the license and the research activities. The Company evaluates whether the deliverables have standalone value. However, since the Company's discovery technologies are highly specialized, the Company has determined that the license does not have standalone value without the ongoing research and development services and accounts for these arrangements as a single unit of accounting.

As a result, the Company has deferred revenue of $7,232,000 and $3,032,000 as of December 31, 2011 and 2012, respectively, related to these arrangements. For the years ended December 31, 2011 and 2012, the Company recognized approximately $6,878,000 and $6,618,000 in collaboration revenue, respectively.

The Company is eligible to receive additional payments from its early stage discovery research arrangements if the discovery compounds are ultimately developed and commercialized. The aggregate potential payments the Company is eligible for if all products are developed is $143 million and up to $252 million in sales milestones upon achievement of specified sales events and up to double digit royalties on worldwide annual net sales of the licensed product.

The Company considers that each of the potential milestone events under the agreement would be substantive because the applicable criteria of its revenue recognition policy (see Note 2) would be satisfied.

Grant revenue

The company receives grant funding from various institutions and governmental bodies. The grants are typically for early discovery research, and typically the grant program lasts from two to five years. The Company records revenue as the research activities are performed. If the granting agency provides for an upfront payment, the amount is deferred and recognized as revenue as the expenditures are incurred.

11. Income taxes

A reconciliation of the U.S. statutory income tax rate to the Company's effective tax rate is as follows:

	December 31	
	2011	2012
Federal income tax (benefit) at statutory rate	34.00%	34.00%
State income tax benefit, net of federal benefit	4.20	3.80
Other	(1.60)	(2.00)
Increase to valuation allowance	(45.90)	(35.80)
Effective income tax rate	(9.3)	0.00%

The Company recognized a tax benefit of $2.3 million in connection with the sale of net operating losses and research and development credits in the New Jersey Transfer Program for the year ended December 31, 2011. The significant components of the Company's deferred tax assets and liabilities at December 31, 2011 and 2012 are as follows:

	2011	2012
Deferred tax assets:		
Amortization	$ 103,355	$ 91,871
Depreciation	1,196,569	1,535,952
Accrued expense	321,925	1,208,846
Deferred revenue	15,738,180	6,962,506
Federal tax credits	5,734,074	5,383,092
State tax credits	1,495,773	1,094,833
Federal net operating losses	56,074,019	71,752,278
State net operating losses	5,024,189	7,619,606
Capitalized research and development costs	11,184,849	10,479,055
Other	600,059	742,341
Total gross deferred tax assets	97,472,992	106,870,380
Less valuation allowance	(97,472,992)	(106,870,380)
Net deferred tax assets	$ —	$ —

At December 31, 2011 and 2012, the Company recorded a full valuation allowance against its net deferred tax assets of approximately $97,473,000 and $106,870,000, respectively. The change in the valuation allowance during the years ended December 31, 2011 and 2012 was approximately $12,909,000 and $9,397,000, respectively. A full valuation allowance has been recorded since, in the judgment of management, these assets are not more likely than not to be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during periods in which those temporary differences and carryforwards become deductible or are utilized.

As of December 31, 2012, the Company has approximately $211,036,000 and $128,276,000 of federal and state net operating loss carryforwards, respectively. As of December 31, 2012, credit carryforwards for federal and state purposes are approximately $5,383,000 and $1,546,000, respectively. The federal net operating loss carryforwards begin to expire in 2021, while the federal credit carryforwards begin to expire in 2013. State net operating loss carryforwards begin to expire in 2029, and the state credit carryforwards begin to expire in 2022. Sections 382 and 383 of the Internal Revenue Code of 1986 subject the future utilization of net operating losses and certain other tax attributes, such as research and experimental tax credits, to an annual limitation in the event of certain ownership changes, as defined. The Company has had a number of equity transactions since inception, and several of these have created ownership changes that could create such a limitation. The Company has not recently performed an analysis to determine the Company's ability to utilize such carryforwards prior to expiration. An analysis will be performed in the future, as necessary.

The State of New Jersey provides the Technology Business Tax Certificate Transfer Program enabling approved unprofitable biotechnology businesses to sell their unused net operating loss carryforwards to unaffiliated, profitable corporate taxpayers in the State of New Jersey for cash. The Company has participated in this program and sold state net operating losses totaling $28,463,900 during 2011. The New Jersey net operating losses sold during 2011 were generated during 2009. For 2011, the Company established a receivable for the $2,305,576, which was received in 2012.

The income tax benefit for the years ended December 31, 2011 and 2012 differed from the amounts computed by applying the U.S. federal income tax rate of 34% to loss before tax benefit as a result of non-deductible expenses, tax credits generated, utilization of net operating loss carryforwards and increases in the Company's valuation allowance. At December 31, 2012 the Company had no unrecognized income tax benefits. The Company applies the accounting guidance for uncertain income tax provisions. This guidance clarifies the accounting for uncertainty in income taxes recognized in financial statements and requires the impact of a tax position to be recognized in the financial statements if that position is more likely than not of being sustained by the taxing authority. As of December 31, 2012, the Company did not have any unrecognized tax benefits and has not accrued any interest or penalties through 2012. The Company does not expect to have any unrecognized tax benefits within the next twelve months. The Company's policy is to recognize interest and penalties related to tax matters within the income tax provision. Tax years beginning in 2009 are generally subject to examination by taxing authorities, although net operating losses from all years are subject to examinations and adjustments for at least three years following the year in which the attributes are used.

12. Commitments and contingencies

Operating leases

The Company leases office space under a noncancelable operating lease through February 2019. Rent expense was approximately $735,000 and $739,000 for the years ended December 31, 2011 and 2012,

respectively. The Company also leases certain office equipment under operating leases. Future minimum lease payments as of December 31, 2012 are as follows:

2013	$ 879,000
2014	849,000
2015	849,000
2016	849,000
2017	849,000
Thereafter	1,113,000
	$ 5,388,000

Other contingencies

Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products. The Company has entered into funding agreements with The Wellcome Trust Limited (Wellcome Trust) for the research and development of small molecule compounds. To the extent that the Company develops and commercializes program intellectual property on a for-profit basis, it may become obligated to pay to Wellcome Trust development and regulatory milestone payments of up to an aggregate of $68.9 million and single-digit royalties on sales of any research program product. The Company's obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that we successfully develop and subsequently commercialize or, if we outlicense rights to a collaboration product, a specified percentage of certain payments we receive from our licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. The Company's obligation to make such payments would end upon our payment to the SMA Foundation of a specified amount.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur.

13. 401(k) plan

The Company maintains a 401(k) plan for its employees. Employee contributions are voluntary. The Company may match employee contributions in amounts to be determined at the Company's sole discretion. The Company provides a 20% matching contribution for up to the first 5% of each contributing employee's base salary contributions. For the years ended December 31, 2011 and 2012, the Company made matching contributions to the 401(k) plan and recorded expense of approximately $177,000 and $154,000, respectively.

14. Subsequent events

On January 29, 2013, the Company entered into a financing arrangement with certain existing investors providing for the issuance by the Company of convertible promissory notes and warrants to purchase shares of Series One and Series Two. The Company issued convertible promissory notes in January and February 2013 in an aggregate principal amount of $6 million under this financing arrangement. This financing was closed in anticipation of an additional financing event.

On March 7, 2013, the Company closed a private placement of a new series of convertible preferred stock that resulted in another recapitalization event. In this private placement, the Company issued and sold an aggregate of 4,497,035 shares of Series Four senior preferred stock (Series Four) for an aggregate purchase price of approximately $54 million. In addition, the Company issued an aggregate of 502,919 shares of Series Four upon conversion of the convertible promissory notes described above that it originally issued in January and February 2013. In connection with this private placement, the Company effected a one-for-120 reverse stock split of its common stock and an exchange of outstanding shares of Series One, Series Two and Series Three into an aggregate of 6,700,487 shares of a new series of Series Five junior preferred stock (Series Five). In addition, the Company issued an aggregate of 2,095,515 shares Series Five upon the automatic exercise of the Series One and Series Two warrants described above that it originally issued in January 2013.

The rights and preferences of the shares of Series Four and Five are as follows:

Dividends—The holders of Series Four and Series Five, in preference to the holders of common stock, are entitled to noncumulative dividends when and if declared by the Board of Directors.

Liquidation—Upon the liquidation, dissolution, reorganization or winding-up of the Company, the holders of Series Four will be entitled to receive, before any distribution or payment is made to any other class of security, an amount equal to the original issuance price, plus all declared, but unpaid, dividends. To the extent there are excess assets to distribute, the holders of Series Five will be entitled to receive, before any distribution or payment is made to the holders of the common stock, an amount equal to the stated liquidation preference, plus all declared, but unpaid, dividends. To the extent there are remaining assets to distribute, the holders of common stock will be entitled to receive such remaining assets.

Voting—Each holder of Series Four and Series Five are entitled to cast the number of votes into which such holder's shares would convert. Except as required by law, holders of common stock have limited voting rights. Additionally, except as required by law, and except in certain enumerated circumstances, holders of Series Four and Series Five shall vote together with the holders of common stock as a single class.

Conversion—Each share of Series Four and Series Five is convertible at any time at the option of the holder into one share of common stock. These conversion ratios are subject to adjustment for certain dilutive events, including certain types of stock splits or stock dividends or future recapitalizations.

On March 5, 2013, the Company's Board of Directors approved the 2013 Stock Incentive Plan, which provides for the granting of stock option awards, stock appreciation rights, restricted stock, restricted stock units and other stock-based in the aggregate of 739,937 shares of Common Stock (calculated after the one-for-120 reverse stock split of its common stock). On March 5, 2013, the Board approved a grant of 735,324 shares of restricted stock and 4,613 stock options. There are no additional shares available for issuance under this plan.

shares

Common stock

Prospectus

J.P. Morgan
Credit Suisse

Cowen and Company
Wedbush Securities

, 2013

Until , 2013, all dealers that buy, sell or trade in our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

Part II

Information not required in prospectus

Item 13. Other expenses of issuance and distribution.

The following table sets forth the expenses to be incurred in connection with the offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimates except the Securities and Exchange Commission's registration fee and the Financial Industry Regulatory Authority, Inc. filing fee.

	Amount
Securities and Exchange Commission registration fee	$ 11,594
Financial Industry Regulatory Authority, Inc. filing fee	13,250
NASDAQ Global Stock Market listing fee	*
Accountants' fees and expenses	*
Legal fees and expenses	*
Blue Sky fees and expenses	*
Transfer Agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous fees and expenses	*
Total expenses	$ *

* To be filed by amendment.

Item 14. Indemnification of directors and officers.

Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of its directors or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. Our certificate of incorporation provides that no director shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the Delaware General Corporation Law prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the General Corporation Law of the State of Delaware provides that a corporation has the power to indemnify a director, officer, employee, or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and

reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

Our certificate of incorporation provides that we will indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of us) by reason of the fact that he or she is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (all such persons being referred to as an "Indemnitee"), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom, if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful. Our certificate of incorporation also provides that we will indemnify any Indemnitee who was or is a party to an action or suit by or in the right of us to procure a judgment in our favor by reason of the fact that the Indemnitee is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise, or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to us, unless a court determines that, despite such adjudication but in view of all of the circumstances, he or she is entitled to indemnification of such expenses. Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by us against all expenses (including attorneys' fees) actually and reasonably incurred by him or her or on his or her behalf in connection therewith. If we do not assume the defense, expenses must be advanced to an Indemnitee under certain circumstances.

We have entered into indemnification agreements with our directors and executive officers. In general, these agreements provide that we will indemnify the director or executive officer to the fullest extent permitted by law for claims arising in his or her capacity as a director or officer of our company or in connection with their service at our request for another corporation or entity. The indemnification agreements also provide for procedures that will apply in the event that a director or executive officer makes a claim for indemnification and establish certain presumptions that are favorable to the director or executive officer.

We maintain a general liability insurance policy which covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

The underwriting agreement we will enter into in connection with the offering of common stock being registered hereby provides that the underwriters will indemnify, under certain conditions, our directors and officers (as well as certain other persons) against certain liabilities arising in connection with such offering.

Item 15. Recent Sales of Unregistered Securities.

Set forth below is information regarding shares of our common stock, shares of our preferred stock, warrants to purchase shares of our preferred stock and promissory notes issued, and stock options and restricted stock awards granted, by us within the past three years that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Also included is the consideration, if any, received by us for such shares and options and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

(a) Issuance of securities

In March 2013, we issued and sold an aggregate of 4,497,035 shares of our series four senior preferred stock, at a price per share of $12.00, for an aggregate purchase price of $53,964,420. In addition, we issued an aggregate of 502,919 shares of our series four senior preferred stock upon conversion of the convertible promissory notes described below that we originally issued in January and February 2013. All outstanding shares of our series four senior preferred stock will automatically convert into an aggregate of 4,999,954 shares of common stock upon the completion of this offering. In connection with the series four senior preferred stock financing, we also effected a reclassification of all of the outstanding shares of our series one preferred stock, series two preferred stock and series three preferred stock into an aggregate of 6,700,487 shares of series five junior preferred stock. In addition, we issued an aggregate of 2,095,515 shares of our series five junior preferred stock upon the automatic exercise of the preferred stock warrants described below that we originally issued in January 2013. All outstanding shares of our series five junior preferred stock will automatically convert into an aggregate of 8,796,002 shares of our common stock upon the completion of this offering.

In January and February 2013, we issued convertible promissory notes in an aggregate principal amount of $6,000,000. These promissory notes converted into an aggregate of 502,919 shares of series four senior preferred stock in connection with the series four senior preferred stock financing in March 2013. In connection with this financing, in January 2013, we issued to holders of the convertible promissory notes described above warrants to purchase an aggregate of 515,186 shares of series one preferred stock, at an exercise price of $0.01 per share, and warrants to purchase an aggregate of 2,012,489 shares of series two preferred stock, at an exercise price of $0.01 per share. In connection with our series four senior preferred stock financing, in March 2013, all outstanding warrants to purchase series one preferred stock and series two preferred stock that were issued in January 2013 were automatically exercised for shares of series five junior preferred stock.

In June and July 2012, we issued an aggregate of 1,483,337 shares of our series one preferred stock, at a price per share of $20.00, for an aggregate purchase price of $29,666,740. In connection with the series one preferred stock financing, we also effected a reclassification of all of our previously outstanding shares of preferred stock into an aggregate of 10,701,405 shares of series two preferred stock and 2,853,517 shares of series three preferred stock. In connection with the series four senior preferred stock financing in March 2013, all outstanding shares of our series one preferred stock, series two preferred stock and series three preferred stock were automatically reclassified into an aggregate of 6,700,487 shares of series five junior preferred stock.

No underwriters were involved in the foregoing issuances of securities. The securities described in this section (a) of Item 15 were issued to accredited investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from

such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(b) Stock option grants

Between January 1, 2010 and March 15, 2013, we issued to certain employees, directors and consultants options to purchase an aggregate of 24,257 shares of our common stock, of which, as of March 15, 2013, options to purchase 5,626 shares of our common stock had been exercised or forfeited, and options to purchase 18,562 shares of our common stock remained outstanding at a weighted-average exercise price of $ per share.

The issuances of stock options and the shares of our common stock issuable upon the exercise of the options described in this paragraph (b) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

(c) Restricted stock grants

In March 2013, we issued an aggregate of 735,324 shares of common stock to our executive officers, directors, advisors and certain other employees in consideration for services provided or to be provided.

The common stock described in this section (c) of Item 15 was issued pursuant to written compensatory plans or arrangements with our employees, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

(d) Issuance of warrants

In connection with our 2013 bridge financing, in January 2013, we issued to holders of the convertible promissory notes described above warrants to purchase an aggregate of 515,186 shares of series one preferred stock, at an exercise price of $0.01 per share, and warrants to purchase an aggregate of 2,012,489 shares of series two preferred stock, at an exercise price of $0.01 per share. In connection with our series four senior preferred stock financing, in March 2013, all outstanding warrants to purchase series one preferred stock and series two preferred stock that were issued in January 2013 were automatically exercised for shares of series five junior preferred stock.

All of the foregoing securities are deemed restricted securities for purposes of the Securities Act. All certificates representing the issued shares of capital stock described in this Item 15 included appropriate legends setting forth that the securities have not been registered and the applicable restrictions on transfer.

Item 16. Exhibits and financial statement schedules.

The exhibits to the registration statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

Item 17. Undertakings.

(a) The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriter to permit prompt delivery to each purchaser.

(b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

(c) The undersigned registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

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Signatures

Pursuant to the requirements of the Securities Act, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of South Plainfield, State of New Jersey, on this day of , 2013.

PTC THERAPEUTICS, INC.

By: _____

Name:
Title:

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Signatures and power of attorney

We, the undersigned officers and directors of PTC Therapeutics, Inc., hereby severally constitute and appoint Stuart W. Peltz and Mark E. Boulding, and each of them singly (with full power to each of them to act alone), our true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution in each of them for him and in his name, place and stead, and in any and all capacities, to sign any and all amendments (including post-effective amendments) to this registration statement (or any other registration statement for the same offering that is to be effective upon filing pursuant to Rule 462(b) under the Securities Act of 1933), and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as full to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this Registration Statement has been signed by the following persons in the capacities held on the dates indicated.

Signature	Title	Date
Stuart W. Peltz, Ph.D.	Chief Executive Officer and Director (principal executive officer)	, 2013
William Hornung	Vice President, Finance and Corporate Controller (principal financial and accounting officer)	, 2013
Michael Schmertzler	Chairman of the Board	, 2013
Richard Aldrich	Director	, 2013
Axel Bolte	Director	, 2013
Allan Jacobson, Ph.D.	Director	, 2013
Adam Koppel, M.D., Ph.D.	Director	, 2013

Signature	Title	Date
Michael Kranda	Director	, 2013
Geoffrey McDonough, M.D.	Director	, 2013
David P. Southwell	Director	, 2013
Jerome B. Zeldis, Ph.D.	Director	, 2013

Exhibit index

Exhibit number	Description of exhibit
1.1*	Underwriting Agreement
3.1	Amended and Restated Certificate of Incorporation of the Registrant
3.2	Amended and Restated Bylaws of the Registrant
3.3*	Form of Certificate of Incorporation of the Registrant (to be effective upon the closing of this offering)
3.4*	Form of Bylaws of the Registrant (to be effective upon the closing of this offering)
4.1*	Specimen Stock Certificate evidencing the shares of common stock
4.2	Amended and Restated Investor Rights Agreement dated as of March 7, 2013
5.1*	Opinion of Wilmer Cutler Pickering Hale and Dorr LLP
10.1	1998 Employee, Director and Consultant Stock Option Plan, as amended
10.2	Form of Incentive Stock Option Agreement under 1998 Employee, Director and Consultant Stock Option Plan
10.3	Form of Nonstatutory Stock Option Agreement under 1998 Employee, Director and Consultant Stock Option Plan
10.4	2009 Equity and Long Term Incentive Plan
10.5	Form of Incentive Stock Option Agreement under 2009 Equity and Long Term Incentive Plan
10.6	Form of Nonstatutory Stock Option Agreement under 2009 Equity and Long Term Incentive Plan
10.7	2013 Stock Incentive Plan
10.8	Form of Restricted Stock Agreement under 2013 Stock Incentive Plan
10.9	Form of Nonstatutory Stock Option Agreement under 2013 Stock Incentive Plan
10.10*	2013 Public Company Stock Incentive Plan
10.11*	Form of Incentive Stock Option Agreement under 2013 Public Company Stock Incentive Plan
10.12*	Form of Nonstatutory Stock Option Agreement under 2013 Public Company Stock Incentive Plan
10.13	Lease Agreement, dated as of July 11, 2000, as amended, between the Registrant and 46.24 Associates L.P.
10.14†	License and Collaboration Agreement, dated as of November 23, 2011, by and among the Registrant, F. Hoffmann-La Roche Ltd, Hoffmann-La Roche Inc. and Spinal Muscular Atrophy Foundation
10.15†	Sponsored Research Agreement, dated as of June 1, 2006, by and between the Registrant and Spinal Muscular Atrophy Foundation
10.16†	Funding Agreement, dated as of May 26, 2010, by and between the Registrant and The Wellcome Trust Limited
10.17†	Funding Agreement by and between the Registrant and The Wellcome Trust Limited, dated as of December 21, 2011

Exhibit number	Description of exhibit
10.18	Loan and Security Agreement, dated as of September 21, 2009, as amended, by and among the Registrant, Oxford Finance Corporation and the Lenders named therein
21.1	Subsidiaries of the Registrant
23.1*	Consent of Ernst & Young LLP independent registered public accounting firm
23.2*	Consent of Wilmer Cutler Pickering Hale and Dorr LLP (included in Exhibit 5.1)
24.1	Power of Attorney (included on signature page)

* To be filed by amendment.

† Confidential treatment requested as to portions of the exhibit. Confidential materials omitted and filed separately with the Securities and Exchange Commission.

AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION
OF
PTC THERAPEUTICS, INC.

(Pursuant to Sections 242 and 245 of the
General Corporation Law of the State of Delaware)

PTC Therapeutics, Inc. a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the "**General Corporation Law**"),

DOES HEREBY CERTIFY:

1. That the name of this corporation is PTC Therapeutics, Inc., and that this corporation was originally incorporated pursuant to the General Corporation Law on March 31, 1998 under the name PTC Therapeutics, Inc. The Certificate of Incorporation was most recently amended on May 25, 2012.

2. That the Board of Directors duly adopted resolutions proposing to amend and restate the Amended and Restated Certificate of Incorporation of this corporation, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

RESOLVED, that the Amended and Restated Certificate of Incorporation of this corporation be amended and restated in its entirety to read as follows:

FIRST: The name of this corporation is PTC Therapeutics, Inc. (the **"Corporation"**).

SECOND: The address of the registered office of the Corporation in the State of Delaware is 2711 Centerville Road, Suite 400, in the City of Wilmington, County of New Castle. The name of its registered agent at such address is Corporation Service Company.

THIRD: The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

FOURTH: The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 17,000,000 shares of Common Stock, $0.001 par value per share ("**Common Stock**"), and (ii) 14,690,000 shares of Preferred Stock, $0.001 par value per share ("**Preferred Stock**"), of which (a) 5,050,000 shares are hereby designated "**Series Four Senior Preferred Stock**" and (b) 9,640,000 shares are hereby designated "**Series Five Junior Preferred Stock**". The Series Four Senior Preferred Stock and Series Five Junior Preferred Stock are collectively referred to herein as the "**Designated Preferred Stock**".

At the time at which the Corporation shall file this Amended and Restated Certificate of Incorporation (the **"Effective Time"**), (i) a one-for 120 reverse stock split of the Corporation's Common Stock (the **"Reverse Stock Split**") shall become effective, pursuant to which each 120 shares of Common Stock outstanding or held in treasury of the Corporation immediately prior to the Effective Time, shall be reclassified and combined into one fully paid and non-assessable share of Common Stock automatically and without any action by the holder thereof and shall represent one share of Common Stock from and after the Effective Time, (ii) each share of the Corporation's previously designated Series One Preferred Stock, par value $0.001 per share (the **"Former Series One Preferred"**), issued and outstanding or held in treasury of the Corporation immediately prior to the Effective Time, shall be automatically reclassified into an amount equal to a fraction, the numerator of which is 430 and the denominator of which is 120, fully paid and nonassessable shares of Series Five Junior Preferred Stock, without any action on the part of the holder thereof; (iii) each share of the Corporation's previously designated Series Two Preferred Stock, par value $0.001 per share (the **"Former Series Two Preferred"**), issued and outstanding or held in treasury of the Corporation immediately prior to the Effective Time, shall be automatically reclassified into an amount equal to a fraction, the numerator of which is 15 and the denominator of which is 120, fully paid and nonassessable shares of Series Five Junior Preferred Stock, without any action on the part of the holder thereof and (iv) each share of the Corporation's previously designated Series Three Preferred Stock, par value $0.001 per share (the **"Former Series Three Preferred"**), issued and outstanding or held in treasury of the Corporation immediately prior to the Effective Time, shall be automatically reclassified into an amount equal to a fraction, the numerator of which is 2 and the denominator of which is 120, fully paid and nonassessable share of Series Five Junior Preferred Stock, without any action on the part of the holder thereof. The Former Series One Preferred, Former Series Two Preferred and Former Series Three Preferred are collectively referred to herein as the **"Former Preferred Stock"**. The reclassification of all such shares of Former Preferred Stock into shares of Series Five Junior Preferred Stock as provided hereby is referred to herein as the **"Recapitalization"**. No fractional shares shall be issued in connection with the Reverse Stock Split or the Recapitalization. Any stockholder who would otherwise be entitled to receive a fractional share of Common Stock as a result of the Reverse Stock Split shall receive in lieu thereof cash in an amount equal to the amount of such fraction multiplied by the then fair market value of a share of Common Stock as determined by the Board of Directors of the Corporation. Any stockholder who would otherwise be entitled to receive a fractional share of Series Five Junior Preferred Stock as a result of the Recapitalization shall receive in lieu thereof cash in an amount equal to the amount of such fraction multiplied by the then fair market value of a share of Series Five Junior Preferred Stock as determined by the Board of Directors of the Corporation. At and after the Effective Time, each outstanding certificate that prior thereto represented shares of Common Stock that were issued and outstanding immediately prior to the Effective Time shall be deemed for all purposes to evidence ownership of and to represent that whole number of shares of Common Stock into which the shares of Common Stock represented by such certificate shall have reclassified and combined as herein provided. At and after the Effective Time, each outstanding certificate that prior thereto represented shares of Former Preferred Stock shall be deemed for all purposes to evidence ownership of and to represent that whole number of shares of Series Five Junior Preferred Stock into which the shares of Former Preferred Stock represented by such certificate shall have been reclassified as herein provided. Each holder of record of a certificate or certificates representing one or more shares of Common Stock that were

issued and outstanding immediately prior to the Effective Time shall be entitled, upon surrender of such certificate or certificates to the Corporation for cancellation, as soon as reasonably practicable, (i) to have made in the Corporation's stock ledger, a book-entry notation or (ii) at the request of such holder, to have issued and delivered to such holder a certificate or certificates, in the case of either clause (i) or (ii), representing the whole number of shares of Common Stock to which such holder shall be entitled pursuant to the provisions of this paragraph. Each holder of record of a certificate or certificates representing one or more shares of Former Preferred Stock shall be entitled, upon surrender of such certificate or certificates to the Corporation for cancellation, as soon as reasonably practicable, (i) to have made in the Corporation's stock ledger a book-entry notation or (ii) at the request of such holder, to have issued and delivered to such holder a certificate or certificates, in in the case of either clause (i) or (ii), representing the whole number of shares of Series Five Junior Preferred Stock to which such holder shall be entitled pursuant to the provisions of this paragraph. No cash in lieu of any fractional share shall be paid to any stockholder until such stockholder shall have surrendered for cancellation or otherwise accounted to the Corporation for the outstanding stock certificates entitling such stockholder to such cash. All share and per share amounts hereinafter set forth in this Amended and Restated Certificate of Incorporation are set forth after giving effect to the Reverse Stock Split and the Recapitalization and no further adjustment thereto shall be made as a result of the Reverse Stock Split or the Recapitalization.

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

A. COMMON STOCK

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth herein.

2. Voting. The holders of the Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders (and written actions in lieu of meetings); provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to the Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the General Corporation Law. There shall be no cumulative voting. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of the Certificate of Incorporation) the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

B. PREFERRED STOCK

The Designated Preferred Stock shall have the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. Unless otherwise indicated, references to "Sections" or "Subsections" in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth.

1. Dividends.

(a) The Corporation shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Corporation (other than dividends on shares of Common Stock payable in shares of Common Stock) unless (in addition to the obtaining of any consents required elsewhere in the Certificate of Incorporation) the holders of the Series Four Senior Preferred Stock and the Series Five Junior Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of such series of Preferred Stock in an amount at least equal to (i) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, that dividend per share of Series Four Senior Preferred or Series Five Junior Preferred Stock, as the case may be, as would equal the product of (A) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (B) the number of shares of Common Stock issuable upon conversion of a share of Series Four Senior Preferred Stock at the then applicable Senior Preferred Conversion Price or Series Five Junior Preferred Stock at the then applicable Junior Preferred Conversion Price, as the case may be, in each case calculated on the record date for determination of holders entitled to receive such dividend or (ii) in the case of a dividend on any class or series that is not convertible into Common Stock, at a rate per share of the applicable series of Preferred Stock determined by (A) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (B) multiplying such fraction by an amount equal to, as applicable (and as defined below), the Senior Preferred Original Issue Price or the Junior Preferred Stated Amount; provided that, if the Corporation declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of the Series Four Senior Preferred Stock and the Series Five Junior Preferred Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest dividend on such series of Preferred Stock.

(b) Notwithstanding the foregoing paragraph (a), the Corporation shall not declare, pay or set aside any dividends on shares of Series Five Junior Preferred Stock unless (in addition to the obtaining of any consents required elsewhere in the Certificate of Incorporation) the holders of the Series Four Senior Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Series Four Senior Preferred Stock in an amount at least equal to the product of (i) the dividend payable on each share of Series Five Junior Preferred Stock determined as if all shares of Series Five Junior Preferred Stock had been converted into Common Stock at the then applicable Junior Preferred Conversion Price and (ii) the number of shares of Common Stock issuable upon conversion of a share of Series Four Senior Preferred Stock at the then applicable Senior Preferred Conversion Price, in

each case calculated on the record date for determination of holders entitled to receive such dividend.

(c) The "**Senior Preferred Original Issue Price**" shall mean $12.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Four Senior Preferred Stock. The "**Junior Preferred Stated Amount**" shall mean $12.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Five Junior Preferred Stock.

2. Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.

2.1 Preferential Payments to Holders of Designated Preferred Stock. Subject to the rights of any other series of Preferred Stock:

2.1.1. Preferential Payments to Holders of Series Four Senior Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Series Four Senior Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders, on a pari passu basis and before any payment shall be made to the holders of Series Five Junior Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share of Series Four Senior Preferred Stock equal to the greater of (i) the Senior Preferred Original Issue Price, plus any dividends declared but unpaid thereon, and (ii) such amount per share as would have been payable had all shares of Designated Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (such greater amount, the "**Senior Preferred Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series Four Senior Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.1, the holders of shares of Series Four Senior Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.1.2. Preferential Payments to Holders of Series Five Junior Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, and only after the payment of all preferential amounts required to be paid to the holders of Series Four Senior Preferred Stock pursuant to Subsection 2.1.1, the holders of shares of Series Five Junior Preferred Stock then outstanding shall be entitled to be paid out of any remaining assets of the Corporation available for distribution to its stockholders, on a pari passu basis and before any payment shall be made to the holders of Common Stock by reason of their ownership thereof, an amount per share of Series Five Junior Preferred Stock equal to the greater of (i) the Junior Preferred Stated Amount, plus any dividends declared but unpaid thereon, and (ii) such amount per share as would have been payable had all shares of Designated Preferred Stock been converted into Common Stock

pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (such greater amount, the "**Junior Preferred Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series Five Junior Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.2, the holders of shares of Series Five Junior Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.2 Distribution of Remaining Assets to Holders of Common Stock. Subject to the rights of any other series of Preferred Stock, in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, and only after the payment of all preferential amounts required to be paid to the holders of shares of Designated Preferred Stock pursuant to Subsection 2.1, the holders of shares of Common Stock then outstanding shall be entitled to receive the remaining assets of the Corporation available for distribution to its stockholders, pro rata based on the number of shares of Common Stock held by each such holder.

2.3 Deemed Liquidation Events.

2.3.1. Definition. Each of the following events shall be considered a "**Deemed Liquidation Event**" unless (i) the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock and (ii) the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock, in the case of each of clause (i) and (ii), acting as separate classes, elect otherwise by written notice sent to the Corporation at least 30 business days prior to the effective date of any such event:

(a) a merger or consolidation in which

(i) the Corporation is a constituent party or

(ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation involving the Corporation or a subsidiary in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock of (1) the surviving or resulting corporation or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all the assets of the Corporation and its subsidiaries taken as a whole, or the sale or disposition (whether by merger or otherwise) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

2.3.2. Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(i) unless the agreement or plan of merger or consolidation for such transaction (the "**Merger Agreement**") provides that the consideration payable to the stockholders of the Corporation shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2.

(b) In the event of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or 2.3.1(b), if the Corporation does not effect a dissolution of the Corporation under the General Corporation Law within 90 days after such Deemed Liquidation Event, then (i) the Corporation shall send a written notice to each holder of Designated Preferred Stock no later than the 90th day after the Deemed Liquidation Event advising such holders of their right (and the requirements to be met to secure such right) pursuant to the terms of the following clause (ii) to require the redemption of such shares of Designated Preferred Stock, and (ii) unless (A) the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock and (B) the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock, in the case of clauses (A) and (B), acting as separate classes, elect in a written instrument delivered to the Corporation not later than 120 days after such Deemed Liquidation Event not to proceed with such redemption, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the Board of Directors of the Corporation), together with any other assets of the Corporation available for distribution to its stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (the **"Available Proceeds**"), to the extent legally available therefor, on the 150th day after such Deemed Liquidation Event (the **"Redemption Date"**), to redeem (i) prior to any redemptions of Series Five Junior Preferred Stock, all outstanding shares of Series Four Senior Preferred Stock at a price per share equal to the Senior Preferred Liquidation Amount and (ii) then, all outstanding shares of Series Five Junior Preferred Stock at a price per share equal to the Junior Preferred Liquidation Amount. Notwithstanding the foregoing, in the event of a redemption pursuant to this Subsection 2.3.2(b), (A) if the Available Proceeds are not sufficient to redeem all outstanding shares of Series Four Senior Preferred Stock, the Corporation shall ratably redeem each holder's shares of Series Four Senior Preferred Stock to the fullest extent of such Available Proceeds, and shall redeem the remaining shares as soon as it may lawfully do so under Delaware law governing distributions to stockholders and (B) if, after redeeming all shares of Series Four Senior Preferred Stock, the Available Proceeds are not sufficient to redeem all outstanding shares of Series Five Junior Preferred Stock, the Corporation shall ratably redeem each holder's shares of Series Five Junior

Preferred Stock to the fullest extent of such Available Proceeds, and shall redeem the remaining shares as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this Subsection 2.3.2(b), the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event or in the ordinary course of business.

(c) The Corporation shall send written notice of any redemption pursuant to Subsection 2.3.2(b) (the **"Redemption Notice"**) to each holder of record of Designated Preferred Stock not less than 25 days prior to the Redemption Date. Each Redemption Notice shall state:

(i) the Redemption Date, the number of shares of each series of Designated Preferred Stock to be redeemed on such Redemption Date and the amount of Available Proceeds to be paid on each series of Designated Preferred Stock on such Redemption Date;

(ii) the date upon which the holder's right (if any) to convert shares terminates (as determined in accordance with Subsection 4.1.2); and

(iii) that the holder is to surrender to the Corporation, in the manner and at the place designated, his, her or its certificate or certificates, if any, representing the shares of Designated Preferred Stock to be redeemed.

On or before the applicable Redemption Date, each holder of shares of Designated Preferred Stock to be redeemed on such Redemption Date, unless such holder has exercised his, her or its right to convert such shares as provided in Section 4, shall surrender the certificate or certificates representing such shares, if any (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated in the Redemption Notice, and thereupon the applicable amount of Available Proceeds to be paid for such shares shall be payable to the order of the person whose name appears on such certificate or certificates as the owner thereof. In the event less than all of the shares of Designated Preferred Stock represented by a certificate are redeemed, a book-entry notation shall promptly be made in the Corporation's stock ledger and confirmation thereof shall be delivered to such holder (or at the request of such owner, a new certificate or certificates shall be issued and delivered to such owner) representing the unredeemed shares of Designated Preferred Stock. If a Redemption Notice shall have been duly given, and if on the applicable Redemption Date the applicable amount of Available Proceeds payable upon redemption of the shares of Designated Preferred Stock to be redeemed on such Redemption Date is paid or tendered for payment or deposited with an independent payment agent so as to be available therefor in a timely manner, then notwithstanding that the certificates, if any, evidencing any of the shares of Designated Preferred Stock so called for redemption shall

not have been surrendered, dividends with respect to such shares of Designated Preferred Stock shall cease to accrue after such Redemption Date and all rights with respect to such shares shall forthwith after the Redemption Date terminate, except only the right of the holders to receive the applicable amount of Available Proceeds without interest upon surrender of their certificate or certificates therefor.

2.3.3. Amount Deemed Paid or Distributed. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any such merger, consolidation, sale, transfer, exclusive license, other disposition or redemption shall be the cash or the value of the property, rights or securities paid or distributed to such holders by the Corporation or the acquiring person, firm or other entity. The value of such property, rights or securities shall be determined in good faith by the Board of Directors of the Corporation.

2.3.4. Allocation of Deferred Payments. In the event of a Deemed Liquidation Event pursuant to Subsection 2.3.1(a)(i), if any portion of the consideration payable to the stockholders of the Corporation is placed into escrow and/or is payable to the stockholders of the Corporation subject to contingencies, then, unless (i) the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock and (ii) the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock, in the case of each of clauses (i) and (ii), acting as separate classes, elect otherwise by written notice sent to the Corporation prior to the effective date of such Deemed Liquidation Event, the Merger Agreement shall provide that (a) the portion of such consideration that is not placed in escrow and not subject to any contingencies (the "**Initial Consideration**") shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event and (b) any additional consideration which becomes payable to the stockholders of the Corporation upon release from escrow or satisfaction of contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 after taking into account the previous payment of the Initial Consideration (or any other payments previously made pursuant to this clause (b)) as part of the same transaction.

3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders of the Corporation (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Series Four Senior Preferred Stock and Series Five Junior Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Series Four Senior Preferred Stock or Series Five Junior Preferred Stock, as the case may be, held by such holder are convertible (based on the then applicable Senior Preferred Conversion Price or Junior Preferred Conversion Price, as the case may be) as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the Certificate of Incorporation, holders of Series Four Senior Preferred Stock and Series Five Junior Preferred Stock shall vote together with the holders of Common Stock as a single class.

3.2 Series Four Senior Preferred Stock Protective Provisions. At any time when at least 500,000 shares of Series Four Senior Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Four Senior Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect:

3.2.1. amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the powers, preferences or rights of the Series Four Senior Preferred Stock (including, without limitation, by amending, altering or repealing any provision of this Section 3.2);

3.2.2. create, or authorize the creation of, or issue or obligate itself to issue shares of, any additional class or series of capital stock unless the same ranks junior to the Series Four Senior Preferred Stock with respect to the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption, or increase the authorized number of shares of Series Four Senior Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock unless the same ranks junior to the Series Four Senior Preferred Stock with respect to the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption;

3.2.3. reclassify, alter or amend any existing security of the Corporation that is pari passu with, or junior to, the Series Four Senior Preferred Stock in respect of the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or, to the extent previously junior, pari passu with the Series Four Senior Preferred Stock in respect of any such right, preference or privilege;

3.2.4. liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any merger or consolidation or any other Deemed Liquidation Event, or consent to any of the foregoing, unless the gross cash proceeds payable to stockholders of the Corporation upon consummation of such liquidation, dissolution, winding up, merger, consolidation or other Deemed Liquidation Event shall equal or exceed $200 million;

3.2.5. purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the Designated Preferred Stock as expressly required hereby, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock, (iii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of

such employment or service at the lower of the original purchase price or the then current fair market value thereof or (iv) as approved by the Board of Directors; or

3.2.6. create, or authorize the creation of, or issue, or authorize the issuance of any indebtedness or capital lease obligation, or permit any subsidiary to take any such action with respect to any indebtedness or capital lease obligation, if the aggregate indebtedness of the Corporation and its subsidiaries for borrowed money following such action would exceed $1.0 million, unless such indebtedness or capital lease obligation has received the prior approval of the Board of Directors.

3.3 Series Five Junior Preferred Stock Protective Provisions. At any time when at least 500,000 shares of Series Five Junior Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Five Junior Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect:

3.3.1. amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the powers, preferences or rights of the Series Five Junior Preferred Stock (including, without limitation, by amending, altering or repealing any provision of this Section 3.3);

3.3.2. create, or authorize the creation of, or issue or obligate itself to issue shares of, any additional class or series of capital stock unless the same ranks junior to the Series Five Junior Preferred Stock with respect to the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption, or increase the authorized number of shares of Series Five Junior Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock unless the same ranks junior to the Series Five Junior Preferred Stock with respect to the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption;

3.3.3. reclassify, alter or amend any existing security of the Corporation that is pari passu with, or junior to, the Series Five Junior Preferred Stock in respect of the distribution of assets in the event of a liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or, to the extent previously junior, pari passu with the Series Five Junior Preferred Stock in respect of any such right, preference or privilege;

3.3.4. purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the

Designated Preferred Stock as expressly required hereby, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock, (iii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then current fair market value thereof or (iv) as approved by the Board of Directors; or

3.3.5. create, or authorize the creation of, or issue, or authorize the issuance of any indebtedness or capital lease obligation, or permit any subsidiary to take any such action with respect to any indebtedness or capital lease obligation, if the aggregate indebtedness of the Corporation and its subsidiaries for borrowed money following such action would exceed $1.0 million, unless such indebtedness or capital lease obligation has received the prior approval of the Board of Directors.

3.4 Deemed Liquidation Event Protective Provision. At any time when at least 1,000,000 shares of Series Five Junior Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Five Junior Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any merger or consolidation or any other Deemed Liquidation Event, or consent to any of the foregoing, unless the gross cash proceeds payable to stockholders of the Corporation upon consummation of such liquidation, dissolution, winding up, merger, consolidation or other Deemed Liquidation Event shall equal or exceed $200 million, without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the holders of shares of Series Five Junior Preferred Stock and Series Four Senior Preferred Stock representing a majority of the votes represented by the then outstanding shares of Series Five Junior Preferred Stock and Series Four Senior Preferred Stock consenting or voting (as the case may be) as a single class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

4. Optional Conversion.

The holders of the Designated Preferred Stock shall have conversion rights as follows (the "**Conversion Rights**"):

4.1 Right to Convert.

4.1.1. Conversion Ratios.

(a) Each share of Series Four Senior Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and nonassessable shares of Common Stock as is determined by dividing the Senior Preferred Original Issue Price by the Senior Preferred Conversion Price (as defined below) in effect at the time of conversion. The **"Senior Preferred Conversion Price"** shall initially be equal to $12.00. Such initial Senior Preferred Conversion Price, and the rate at which shares of

Series Four Senior Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below.

(b) Each share of Series Five Junior Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and nonassessable shares of Common Stock as is determined by dividing the Junior Preferred Stated Amount by the Junior Preferred Conversion Price (as defined below) in effect at the time of conversion. The **"Junior Preferred Conversion Price"** shall initially be equal to $12.00. Such initial Junior Preferred Conversion Price, and the rate at which shares of Series Five Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below.

4.1.2. Termination of Conversion Rights. In the event of a liquidation, dissolution or winding up of the Corporation or a Deemed Liquidation Event, the Conversion Rights shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Designated Preferred Stock.

4.2 Fractional Shares. No fractional shares of Common Stock shall be issued upon conversion of the Designated Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock as determined in good faith by the Board of Directors of the Corporation. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Designated Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

4.3 Mechanics of Conversion.

4.3.1. Notice of Conversion. In order for a registered holder of Series Four Senior Preferred Stock or Series Five Junior Preferred Stock to voluntarily convert shares of such series of Preferred Stock into shares of Common Stock, such holder shall provide written notice that such holder elects to convert all or any number of the shares of the Preferred Stock and, if applicable, any event on which such conversion is contingent (and, if such shares of Preferred Stock are then represented in certificated form, surrender the certificate or certificates for such shares of Preferred Stock (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for the Designated Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer

agent)). Such notice shall state such holder's name or the names of the nominees in which such holder wishes the shares of Common Stock (and any requested certificate or certificates representing such Shares) to be issued. If required by the Corporation, certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in

writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer agent) of such notice (and, in the case of shares of Preferred Stock then represented in certificated form, the applicable certificates (or lost certificate affidavit and agreement)) shall be the time of conversion (the "**Conversion Time**"), and the shares of Common Stock issuable upon conversion of such shares of Preferred Stock shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time, (i) deliver to such holder of Preferred Stock, or to his, her or its nominees, confirmation that the Corporation's stock ledger has been notated to reflect (or, at the request of such holder, a certificate or certificates reflecting) the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and confirmation that the Corporation's stock ledger has been notated to reflect (or, at the request of such holder, a certificate or certificates reflecting) the number (if any) of the shares of Preferred Stock represented by the surrendered certificate, if any, that were not converted into Common Stock, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2. Reservation of Shares. The Corporation shall at all times when the Designated Preferred Stock shall be outstanding, reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of the Designated Preferred Stock, such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding Designated Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of the Designated Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to the Certificate of Incorporation. Before taking any action which would cause an adjustment reducing the Senior Preferred Conversion Price or the Junior Preferred Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of the applicable series of Designated Preferred Stock, the Corporation will take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and nonassessable shares of Common Stock at such adjusted Senior Preferred Conversion Price or Junior Preferred Conversion Price, as the case may be.

4.3.3. Effect of Conversion. All shares of Designated Preferred Stock which shall have been surrendered for conversion as herein provided shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive shares of Common Stock in exchange therefor, to receive payment in lieu of any fraction of a share otherwise issuable upon such conversion as provided in Subsection 4.2 and to receive payment of any dividends declared but unpaid thereon. Any shares of any series of Designated Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of such series of Preferred Stock accordingly.

4.3.4. No Further Adjustment. Upon any such conversion, no adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price shall be made for any declared but unpaid dividends on the Series Four Senior Preferred Stock or Series Five Junior Preferred Stock, as the case may be, surrendered for conversion or on the Common Stock delivered upon conversion.

4.3.5. Taxes. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Designated Preferred Stock pursuant to this Section 4. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Designated Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

4.4 Adjustments to Conversion Prices for Diluting Issues.

4.4.1. Special Definitions. For purposes of this Article Fourth, the following definitions shall apply:

(a) "**Option**" shall mean rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or Convertible Securities.

(b) "**Convertible Securities**" shall mean any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.

(c) "**Series Four Original Issue Date**" shall mean the date on which the first share of Series Four Senior Preferred Stock was issued.

(d) "**Additional Shares of Common Stock**" shall mean all shares of Common Stock issued (or, pursuant to Subsection 4.4.3 below, deemed to be issued) by the Corporation after the Series Four Original Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "**Exempted Securities**"):

(i) shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on all series of Designated Preferred Stock on a pro rata basis;

(ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by Subsection 4.5, 4.6,

4.7 or 4.8;

(iii) up to, in the aggregate, that number of shares of Common Stock or Options equal to 15% of the aggregate number of shares of outstanding Common Stock on an as-converted, fully-diluted basis (after giving effect to the issuance of the Series Four Senior Preferred Stock, the Reverse Stock Split and the Recapitalization) measured as of the Series Four Original Issue Date (subject to equitable adjustment in the event of any stock dividend, stock split, combination, reorganization, recapitalization or similar event involving a change in the Common Stock) issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries pursuant to a plan, agreement or arrangement approved by the Board of Directors of the Corporation;

(iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options, or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security;

(v) up to, in the aggregate, that number of shares of Common Stock or Options equal to 1% of the aggregate number of shares of outstanding Common Stock on an as-converted, fully-diluted basis (after giving effect to the issuance of the Series Four Senior Preferred Stock, the Reverse Stock Split and the Recapitalization) measured as of the Series Four Original Issue Date (subject to equitable adjustment in the event of any stock dividend, stock split, combination, reorganization, recapitalization or similar event involving a change in the Common Stock) to institutional lenders in connection with the establishment or maintenance by the Corporation of credit facilities, including equipment lease facilities, approved in each case by the Board of Directors of the Corporation;

(vi) securities issued pursuant to a registered public offering, the closing of which is on or after the Series Four Original Issue Date;

(vii) up to, in the aggregate, that number of shares of Common Stock or Options equal to 1% of the aggregate number of shares of outstanding Common Stock on an as-converted, fully-diluted basis (after giving effect to the issuance of the Series Four Senior Preferred Stock, the Reverse Stock Split and the Recapitalization) measured as of the Series Four Original Issue Date (subject to equitable adjustment in the event of any stock dividend, stock split, combination, reorganization, recapitalization or similar event involving a change in the Common Stock) to any licensor of technology or patent rights to the Corporation or to any collaborative partner or licensee with respect to the development or commercialization of products;

(viii) up to, in the aggregate, that number of shares of Common Stock or Options equal to 4% of the aggregate number of shares of outstanding Common Stock on an as-converted, fully-diluted basis (after giving effect to the issuance of the Series Four Senior Preferred Stock, the Reverse Stock Split and the Recapitalization) measured as of the Series Four Original Issue Date (subject to equitable adjustment in the event of any stock dividend, stock split, combination, reorganization, recapitalization or similar event involving a change in the Common Stock) in connection with the acquisition of another business entity by merger, purchase of all or substantially all of its assets or acquisition of all or substantially all of the equity interest of such business entity;

(ix) shares of Series Four Senior Preferred Stock and Series Five Junior Preferred Stock (and any Common Stock issued or issuable upon conversion thereof) issued in accordance with the terms of the Subscription Agreement, dated on or about the Series Four Original

Issue Date, among the Corporation and the other signatories thereto, or in connection with the Reverse Stock Split or the Recapitalization, as the case may be; or

(x) up to, in the aggregate, 740,000 shares of Common Stock, Options or Convertible Securities (with the number of Convertible Securities issued pursuant to this subparagraph (x) determined based on the number of shares of Common Stock into which such Convertible Securities are convertible or for which such Convertible Securities are exchangeable) (subject to equitable adjustment in the event of any stock dividend, stock split, combination, reorganization, recapitalization or similar event involving a change in the Common Stock) issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries, in consideration of the termination of the PTC Therapeutics, Inc. 2012 Carve-Out Incentive Plan.

4.4.2. No Adjustment of Conversion Prices.

(a) No adjustment in the Senior Preferred Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock.

(b) No adjustment in the Junior Preferred Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock.

4.4.3. Deemed Issue of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Series Four Original Issue Date shall issue any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or shall fix a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock

(as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, the conversion or exchange of such Convertible Securities, shall be deemed to be Additional Shares of Common Stock issued as of the time of such issue or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price pursuant to the terms of Subsection 4.4.4, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (2) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, computed upon the original issue of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to such Senior Preferred Conversion Price or Junior Preferred Conversion Price, as the case may be, as would have obtained had such revised terms been in effect upon the original date of issuance of such Option or Convertible Security. Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, to an amount which exceeds the lower of (i) the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, in effect immediately prior to the original adjustment made as a result of the issuance of such Option or Convertible Security, or (ii) the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date and such readjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price pursuant to the terms of Subsection 4.4.4 (either because the consideration per share (determined pursuant to Subsection 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, then in effect, or because such Option or Convertible Security was issued before the Series Four Original Issue Date), are revised after the Series Four Original Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase in the number of shares of Common Stock issuable upon the exercise, conversion or exchange of any such Option

or Convertible Security or (2) any decrease in the consideration payable to the Corporation upon such exercise, conversion or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Subsection 4.4.3(a) shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option or unconverted or unexchanged Convertible Security (or portion thereof) that resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price pursuant to the terms of Subsection 4.4.4, the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, shall be readjusted to such Senior Preferred Conversion Price or Junior Preferred Conversion Price, as the case may be, as would have obtained had such Option or Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price provided for in this Subsection 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Subsection 4.4.3). If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price that would result under the terms of this Subsection 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, that such issuance or amendment took place at the time such calculation can first be made.

4.4.4. Adjustment of Conversion Prices Upon Issuance of Additional Shares of Common Stock. In the event the Corporation shall at any time after the Series Four Original Issue Date issue Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be issued pursuant to Subsection 4.4.3), without consideration or for a consideration per share less than the Senior Preferred Conversion Price or the Junior Preferred Conversion Price in effect immediately prior to such issue, then the Senior Preferred Conversion Price or the Junior Preferred Conversion Price,

as the case may be, shall be reduced, concurrently with such issue, to a price (calculated to the nearest one-hundredth of a cent) determined in accordance with the following formula:

$$CP_2 = CP_1 * (A + B) \div (A + C).$$

For purposes of the foregoing formula, the following definitions shall apply:

(i) "CP_2" shall mean the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, in effect immediately after such issuance of Additional Shares of Common Stock;

(ii) "CP_1" shall mean the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, in effect immediately prior to such issuance of Additional Shares of Common Stock;

(iii) "A" shall mean the number of shares of Common Stock outstanding immediately prior to such issuance of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon exercise of Options outstanding immediately prior to such issuance or upon conversion or exchange of Convertible Securities (including the assumed conversion of the Designated Preferred Stock at the then applicable Basic Conversion Price for the applicable series of Designated Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issue);

(iv) "B" shall mean the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued at a price per share equal to CP_1 (determined by dividing the aggregate consideration received by the Corporation in respect of such issuance by CP_1); and

(v) "C" shall mean the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5. Determination of Consideration. For purposes of this Subsection 4.4, the consideration received by the Corporation for the issuance of any Additional Shares of Common Stock shall be computed as follows:

(a) Cash and Property: Such consideration shall:

(i) insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;

(ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issuance, as determined in good faith by the Board of Directors of the Corporation; and

(iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board of Directors of the Corporation.

(b) Options and Convertible Securities. The consideration per share received by the Corporation for Additional Shares of Common Stock deemed to have been issued pursuant to Subsection 4.4.3, relating to Options and Convertible Securities, shall be determined by dividing

(i) the total amount, if any, received or receivable by the Corporation as consideration for the issuance of such Options or Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration) payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities, by

(ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such

Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities.

4.4.6. Multiple Closing Dates. In the event the Corporation shall issue on more than one date Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to the Senior Preferred Conversion Price or the Junior Preferred Conversion Price pursuant to the terms of Subsection 4.4.4, and such issuance dates occur within a period of no more than 90 days from the first such issuance to the final such issuance, then, upon the final such issuance, the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as

the case may be, shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation shall at any time or from time to time after the Series Four Original Issue Date effect a subdivision of the outstanding Common Stock, the Senior Preferred Conversion Price and the Junior Preferred Conversion Price in effect immediately before that subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation shall at any time or from time to time after the Series Four Original Issue Date combine the outstanding shares of Common Stock, the Senior Preferred Conversion Price and the Junior Preferred Conversion Price in effect immediately before such combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this subsection shall become effective at the close of business on the date the subdivision or combination becomes effective.

4.6 Adjustment for Certain Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series Four Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable on the Common Stock in additional shares of Common Stock, then and in each such event the Senior Preferred Conversion Price and the Junior Preferred Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date, and

(2) the denominator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date plus the number of shares of Common Stock issuable in payment of such dividend or distribution.

Notwithstanding the foregoing, (a) if such record date shall have been fixed and such dividend is not fully paid or if such distribution is not fully made on the date fixed therefor, the Senior Preferred Conversion Price and the Junior Preferred Conversion Price shall be recomputed accordingly as of the close of business on such record date and thereafter the Senior Preferred Conversion Price and the Junior Preferred Conversion Price shall be adjusted pursuant to this subsection as of the time of actual payment of such dividends or distributions; and (b) no such adjustment shall be made if the holders of Series Four Senior Preferred Stock or Series Five Junior Preferred Stock, as the case may be, simultaneously receive a dividend or other distribution of shares of Common Stock in a number equal to the number of shares of Common Stock as they would have received if all outstanding shares of Series Four Senior Preferred Stock or Series Five Junior Preferred Stock, as the case may be, had been converted into Common Stock on the date of such event at the then applicable Basic Conversion Price for the applicable series of Preferred Stock.

4.7 Adjustments for Other Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series Four Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a distribution of shares of Common Stock in respect of outstanding shares of Common Stock) or in other property and the provisions of Section 1 do not apply to such dividend or distribution, then and in each such event the holders of Designated Preferred Stock shall receive, simultaneously with the distribution to the holders of Common Stock, a dividend or other distribution of such securities or other property in an amount equal to the amount of such securities or other property as they would have received if all outstanding shares of Designated Preferred Stock had been converted into Common Stock on the date of such event at the then applicable Basic Conversion Price for the applicable series of Preferred Stock.

4.8 Adjustment for Merger or Reorganization, etc. Subject to the provisions of Subsection 2.3, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which the Common Stock (but not the Designated Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by Subsections 4.4, 4.6 or 4.7), then, following any such reorganization, recapitalization, reclassification, consolidation or merger, each share of Designated Preferred Stock shall thereafter be convertible in lieu of the Common Stock into which it was convertible prior to such event into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock of the Corporation issuable upon conversion of one share of the applicable series of Designated Preferred Stock immediately prior to such reorganization, recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board of Directors of the Corporation) shall be made in the application of the provisions in this Section 4 with respect to the rights and interests thereafter of the holders of the Designated Preferred Stock, to

the end that the provisions set forth in this Section 4 (including provisions with respect to changes in and other adjustments of the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of the applicable series of Designated Preferred Stock.

4.9 Certificate as to Adjustments. Upon the occurrence of each adjustment or readjustment of the Senior Preferred Conversion Price or the Junior Preferred Conversion Price pursuant to this Section 4, the Corporation at its expense shall, as promptly as reasonably practicable but in any event not later than 30 days thereafter, compute such adjustment or readjustment in accordance with the terms hereof and furnish to each holder of each affected series of Designated Preferred Stock a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which such affected series is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. The Corporation shall, as promptly as reasonably practicable after the written request at any time of any holder of Designated Preferred Stock (but in any event not later than 30 days thereafter), furnish or cause to be furnished to such holder a certificate setting forth (i) the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, then in effect, and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property that then would be received upon the conversion of the applicable series of Designated Preferred Stock.

4.10 Notice of Record Date. In the event:

(a) the Corporation shall take a record of the holders of its Common Stock (or other capital stock or securities at the time issuable upon conversion of the Designated Preferred Stock) for the purpose of entitling or enabling them to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, or to receive any other security; or

(b) of any capital reorganization of the Corporation, any reclassification of the Common Stock of the Corporation, or any Deemed Liquidation Event; or

(c) of the voluntary or involuntary dissolution, liquidation or winding-up of the Corporation,

then, and in each such case, the Corporation will send or cause to be sent to the holders of the Designated Preferred Stock a notice specifying, as the case may be, (i) the record date for such dividend, distribution or right, and the amount and character of such dividend, distribution or right, or (ii) the effective date on which such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up is proposed to take place, and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of the Designated Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up, and the amount per share

and character of such exchange applicable to the Designated Preferred Stock and the Common Stock. Such notice shall be sent at least 10 days prior to the record date or effective date for the event specified in such notice.

5. Mandatory Conversion.

5.1 Trigger Events. Upon either (a) the closing of a Qualified Initial Public Offering or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock, together with the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock, voting or consenting as separate classes (the time of such closing or the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the "**Mandatory Conversion Time**"), (i) all outstanding shares of Designated Preferred Stock shall automatically be converted into shares of Common Stock at the Senior Preferred Conversion Price or the Junior Preferred Conversion Price, as the case may be, in effect immediately prior to the Mandatory Conversion Time; and (ii) such shares may not be reissued by the Corporation. A "**Qualified Initial Public Offering**" shall mean (a) the sale of shares of Common Stock in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended (the "**Securities Act**"), resulting in at least $85 million of gross proceeds (before deducting underwriting discounts and offering expenses) or (b) any other firm-commitment underwritten public offering of shares of Common Stock deemed to be a Qualified Initial Public Offering by the vote or written consent of the holders of a majority of the then outstanding shares of Series Four Senior Preferred Stock and the holders of a majority of the then outstanding shares of Series Five Junior Preferred Stock, voting or consenting as separate classes.

5.2 Procedural Requirements. All holders of record of shares of Designated Preferred Stock shall be sent written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all such shares of Designated Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time. Upon receipt of such notice, each holder of shares of Designated Preferred Stock shall surrender his, her or its certificate or certificates for all such shares, if any (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation at the place designated in such notice. If so required by the Corporation, certificates surrendered for conversion shall be endorsed or accompanied by written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or by his, her or its attorney duly authorized in writing. All rights with respect to the Designated Preferred Stock converted pursuant to Subsection 5.1, including the rights, if any, to receive notices and vote (other than as a holder of Common Stock), will terminate at the Mandatory Conversion Time (notwithstanding the failure of the holder or holders thereof to surrender certificates at or prior to such time), except only the rights of the holders thereof, upon surrender of their certificate or certificates, if any (or lost certificate affidavit and agreement) therefor, to receive the items provided for in the next sentence of this Subsection 5.2. As soon as practicable after the Mandatory Conversion Time and the surrender of the certificate or certificates, if any (or lost

certificate affidavit and agreement) for Designated Preferred Stock, the Corporation shall deliver to such holder, or to his, her or its nominees, confirmation that the Corporation's stock ledger has been notated to reflect (or, at the request of such holder, a certificate or certificates reflecting) the number of full shares of Common Stock issuable on such conversion in accordance with the provisions hereof, together with cash as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and the payment of any declared but unpaid dividends on the shares of Designated Preferred Stock converted. Such converted Designated Preferred Stock shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

6. Redeemed or Otherwise Acquired Shares. Any shares of Designated Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Designated Preferred Stock following redemption.

7. Waiver. Any of the rights, powers, preferences and other terms of any series of Designated Preferred Stock set forth herein may be waived on behalf of all holders of such series by the affirmative written consent or vote of the holders of a majority of the then outstanding shares of such series.

8. Notices. Any notice required or permitted by the provisions of this Article Fourth to be given to a holder of shares of Designated Preferred Stock shall be mailed, postage prepaid, to the post office address last shown on the records of the Corporation, or given by electronic communication in compliance with the provisions of the General Corporation Law, and shall be deemed sent upon such mailing or electronic transmission.

FIFTH: Subject to any additional vote required by the Certificate of Incorporation or Bylaws, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the Bylaws of the Corporation.

SIXTH: Subject to any additional vote required by the Certificate of Incorporation, the number of directors of the Corporation shall be determined in the manner set forth in the Bylaws of the Corporation.

SEVENTH: This Article SEVENTH is inserted for the management of the business and for the conduct of the affairs of the Corporation.

1. Elections of Directors. Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.

2. Classes of Directors. The Board of Directors shall be and is divided into three classes, designated Class I, Class II and Class III. Each class shall consist, as nearly as may be possible, of one-third of the total number of directors constituting the entire Board of Directors.

The Board of Directors is authorized to assign members of the Board of Directors already in office to Class I, Class II or Class III at the time such classification becomes effective.

3. Terms of Office. Each director shall serve for a term ending on the date of the third annual meeting of stockholders following the annual meeting of stockholders at which such director was elected; provided that each director initially assigned to Class I shall serve for a term expiring at the Corporation's first annual meeting of stockholders held after the effectiveness of this Amended and Restated Certificate of Incorporation; each director initially assigned to Class II shall serve for a term expiring at the Corporation's second annual meeting of stockholders held after the effectiveness of this Amended and Restated Certificate of Incorporation; and each director initially assigned to Class III shall serve for a term expiring at the Corporation's third annual meeting of stockholders held after the effectiveness of this Amended and Restated Certificate of Incorporation; provided further, that the term of each director shall continue until the election and qualification of his or her successor and be subject to his or her earlier death, resignation or removal.

4. Removal. Directors of the Corporation may be removed either for cause or without cause by the affirmative vote of the holders of at least a majority of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors.

EIGHTH: Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws of the Corporation may provide. The books of the Corporation may be kept outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws of the Corporation.

NINTH: To the fullest extent permitted by law, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director. If the General Corporation Law or any other law of the State of Delaware is amended after approval by the stockholders of this Article Ninth to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any repeal or modification of the foregoing provisions of this Article Ninth by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

TENTH: The following indemnification provisions shall apply to the persons enumerated below.

1. Right to Indemnification of Directors and Officers. The Corporation shall indemnify and hold harmless, to the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, any person (an **"Indemnified Person"**) who was or is made or is threatened to be made a party or is otherwise involved in any action, suit or proceeding, whether civil, criminal, administrative or investigative (a **"Proceeding"**), by reason of the fact

that such person, or a person for whom such person is the legal representative, is or was a director or officer of the Corporation or, while a director or officer of the Corporation, is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation or of a partnership, joint venture, limited liability company, trust, enterprise or nonprofit entity, including service with respect to employee benefit plans, against all liability and loss suffered and expenses (including attorneys' fees) reasonably incurred by such Indemnified Person in such Proceeding. Notwithstanding the preceding sentence, except as otherwise provided in Section 3 of this Article Tenth, the Corporation shall be required to indemnify an Indemnified Person in connection with a Proceeding (or part thereof) commenced by such Indemnified Person only if the commencement of such Proceeding (or part thereof) by the Indemnified Person was authorized in advance by the Board of Directors.

2. Prepayment of Expenses of Directors and Officers. The Corporation shall pay the expenses (including attorneys' fees) incurred by an Indemnified Person in defending any Proceeding in advance of its final disposition, provided, however, that, to the extent required by law, such payment of expenses in advance of the final disposition of the Proceeding shall be made only upon receipt of an undertaking by the Indemnified Person to repay all amounts advanced if it should be ultimately determined that the Indemnified Person is not entitled to be indemnified under this Article Tenth or otherwise.

3. Claims by Directors and Officers. If a claim for indemnification or advancement of expenses under this Article Tenth is not paid in full within 30 days after a written claim therefor by the Indemnified Person has been received by the Corporation, the Indemnified Person may file suit to recover the unpaid amount of such claim and, if successful in whole or in part, shall be entitled to be paid the expense of prosecuting such claim. In any such action the Corporation shall have the burden of proving that the Indemnified Person is not entitled to the requested indemnification or advancement of expenses under applicable law.

4. Indemnification of Employees and Agents. The Corporation may indemnify and advance expenses to any person who was or is made or is threatened to be made or is otherwise involved in any Proceeding by reason of the fact that such person, or a person for whom such person is the legal representative, is or was an employee or agent of the Corporation or, while an employee or agent of the Corporation, is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation or of a partnership, joint venture, limited liability company, trust, enterprise or nonprofit entity, including service with respect to employee benefit plans, against all liability and loss suffered and expenses (including attorneys' fees) reasonably incurred by such person in connection with such Proceeding. The ultimate determination of entitlement to indemnification of persons who are non-director or officer employees or agents shall be made in such manner as is determined by the Board of Directors in its sole discretion. Notwithstanding the foregoing sentence, the Corporation shall not be required to indemnify a person in connection with a Proceeding initiated by such person if the Proceeding was not authorized in advance by the Board of Directors.

5. Advancement of Expenses of Employees and Agents. The Corporation may pay the expenses (including attorneys' fees) incurred by an employee or agent in defending any Proceeding in advance of its final disposition on such terms and conditions as may be determined by the Board of Directors.

6. Non-Exclusivity of Rights. The rights conferred on any person by this Article Tenth shall not be exclusive of any other rights which such person may have or hereafter acquire under any statute, provision of the Certificate of Incorporation, Bylaws of the Corporation, agreement, vote of stockholders or disinterested directors or otherwise.

7. Other Indemnification. The Corporation's obligation, if any, to indemnify any person who was or is serving at its request as a director, officer or employee of another corporation, partnership, limited liability company, joint venture, trust, organization or other enterprise shall be reduced by any amount such person may collect as indemnification from such other corporation, partnership, limited liability company, joint venture, trust, organization or other enterprise.

8. Insurance. The Board of Directors may, to the full extent permitted by applicable law as it presently exists, or may hereafter be amended from time to time, authorize an appropriate officer or officers to purchase and maintain at the Corporation's expense insurance: (a) to indemnify the Corporation for any obligation which it incurs as a result of the indemnification of directors, officers and employees under the provisions of this Article Tenth; and (b) to indemnify or insure directors, officers and employees against liability in instances in which they may not otherwise be indemnified by the Corporation under the provisions of this Article Tenth.

9. Amendment or Repeal. Any repeal or modification of the foregoing provisions of this Article Tenth shall not adversely affect any right or protection hereunder of any person in respect of any act or omission occurring prior to the time of such repeal or modification. The rights provided hereunder shall inure to the benefit of any Indemnified Person and such person's heirs, executors and administrators.

ELEVENTH: The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An "**Excluded Opportunity**" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of, (i) any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, or (ii) any holder of Designated Preferred Stock or any partner, member, director, stockholder, employee or agent of any such holder, other than someone who is an employee of the Corporation or any of its subsidiaries (the persons described in clauses (i) and (ii), collectively, "**Covered Persons**"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person's capacity as a director of the Corporation.

TWELFTH: Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery in the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law or the Certificate of Incorporation or Bylaws of the Corporation or (iv) any action asserting a claim governed by the internal affairs doctrine.

* * *

3. That the foregoing amendment and restatement was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the General Corporation Law.

4. That this Amended and Restated Certificate of Incorporation, which restates and integrates and further amends the provisions of this corporation's Amended and Restated Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

IN WITNESS WHEREOF, this Amended and Restated Certificate of Incorporation has been executed by a duly authorized officer of this corporation on this 7th day of March, 2013.

By: /s/ Stuart Peltz
Name: Stuart W. Peltz
Title: Chief Executive Officer

SECOND AMENDED AND RESTATED BYLAWS

of

PTC THERAPEUTICS, INC.

ARTICLE I
STOCKHOLDERS

Section 1. Annual Meeting.

An annual meeting of the stockholders for the election of directors to succeed those whose terms expire and for the transaction of such other business as may properly come before the meeting shall be held at ten o'clock a.m. or such other time as is determined by the Board of Directors, on such date (other than a Saturday, Sunday or legal holiday) as is determined by the Board of Directors.

Section 2. Special Meetings.

Subject to the rights of the holders of any class or series of preferred stock of the Corporation, special meetings of stockholders of the Corporation may be called only by the Board of Directors pursuant to a resolution adopted by a majority of the total number of directors. Special meetings of the stockholders may be held at such place, if any, within or without the State of Delaware as may be stated in such resolution. Business transacted at any special meeting of stockholders shall be limited to matters relating to the purpose or purposes stated in the notice of meeting.

Section 3. Notice and Place of Meetings.

Written notice of the place, if any, date and time of all meetings of the stockholders, whether annual or special, and the means of remote communications, if any, by which the stockholders and proxyholders may be deemed to be present and vote at such meeting shall be given not less than ten (10) nor more than sixty (60) days before the date on which the meeting is to be held to each stockholder entitled to vote at such meeting, except as otherwise provided herein or in the Certificate of Incorporation of the Corporation (as the same may be amended or restated from time to time, the "Certificate of Incorporation") or required by law. Without limiting the manner by which notice otherwise may be given to the stockholders, any notice shall be effective if given by a form of electronic transmission consented to (in a manner consistent with the General Corporation Law of the State of Delaware) by the stockholder to whom the notice is given. The notice of a special meeting shall state, in addition, the purpose or purposes for which the meeting is called. If notice is given by mail, such notice shall be deemed given when deposited in the United States mail, postage prepaid, directed to the stockholder at such stockholder's address as it appears on the records of the Corporation. If notice is given by electronic transmission, such notice shall be deemed given at the time specified in Section 232 of the General Corporation Law of the State of Delaware.

When a meeting is adjourned to another place, date or time, written notice need not be given of the adjourned meeting if the place, if any, date and time thereof and the means of remote communications, if any are announced at the meeting at which the adjournment is taken;

provided, *however*, that if the date of any adjourned meeting is more than thirty (30) days after the date for which the meeting was originally noticed, or if a new record date is fixed for the adjourned meeting, written notice of the place, if any, date, and time of the adjourned meeting and the means of remote communications, if any, shall be given in conformity herewith. At any adjourned meeting, any business may be transacted that may have been transacted at the original meeting.

All meetings of stockholders, whether annual or special, shall be held at such place as the Board of Directors shall designate or, if not so designated, at the principal office of the Corporation. The Board of Directors may, in its sole discretion, determine that a meeting shall not be held at any place, but may instead be held solely by means of remote communication in a manner consistent with the General Corporation Law of the State of Delaware.

Section 4. Quorum.

At any meeting of the stockholders, the holders of a majority of all of the shares of the stock entitled to vote at the meeting, present in person, present by means of remote communication in a manner, if any, authorized by the Board of Directors in its sole discretion or represented by proxy, shall constitute a quorum for all purposes, unless or except to the extent that the presence of a larger number may be required by law or the Certificate of Incorporation. Except as otherwise provided in the Certificate of Incorporation, where a separate vote by a class or classes of stock is required, a majority of the shares of such class or classes of stock present in person, present by means of remote communication in a manner, if any, authorized by the Board of Directors in its sole discretion or represented by proxy shall constitute a quorum entitled to take action with respect to that vote on that matter. A quorum, once established at a meeting, shall not be broken by the withdrawal of enough votes to leave less than a quorum.

If a quorum shall fail to attend any meeting, the chairman of the meeting or the holders of a majority of the shares of stock entitled to vote who are present in person, present by means of remote communication or represented by proxy, may adjourn the meeting to another place, date, or time.

Section 5. Conduct of Business.

The Chairman of the Board of Directors or his or her designee or, if neither the Chairman of the Board of Directors nor his or her designee is present at the meeting, then a person appointed by a majority of the Board of Directors, shall preside at, and act as chairman of, any meeting of the stockholders. The chairman of any meeting of stockholders shall determine the order of business and the procedures at the meeting, including such regulation of the manner of voting and the conduct of discussion as he or she deems to be appropriate. The Secretary shall act as secretary of the meeting, but in the Secretary's absence, the chairman of the meeting may appoint any person to act as secretary of the meeting.

Section 6. Proxies and Voting.

At any meeting of the stockholders, every stockholder entitled to vote may vote in person (including by means of remote communications, if any, by which stockholders may be deemed to be present in person and vote at such meeting) or by proxy authorized by an instrument executed

or transmitted in a manner permitted by the General Corporation Law of the State of Delaware by the stockholder or such stockholder's agent and delivered (including by electronic transmission) to the Secretary of the Corporation. No such proxy shall be voted or acted upon after three years from the date of its execution, unless the proxy expressly provides for a longer period.

Each stockholder shall have one (1) vote for every share of stock entitled to vote that is registered in his or her name on the record date for the meeting, except as otherwise provided herein, in the Certificate of Incorporation or required by law.

All voting, including on the election of directors but excepting where otherwise required by law, may be by a voice vote; *provided*, *however*, that upon demand therefore by a stockholder entitled to vote or his or her proxy, a vote by ballot shall be taken.

Elections shall be determined in accordance with the voting provisions set forth in the Corporation's Certificate of Incorporation and, to the extent applicable, in the Second Amended and Restated Voting Agreement by and among the Corporation and the stockholders who are signatories thereto, dated as of March 7, 2013 (as the same may be amended or restated from time to time, the "Voting Agreement"). Except as otherwise provided in the Corporation's Certificate of Incorporation, the Voting Agreement (to the extent applicable) or required by law, all other matters shall be determined by a majority of the votes cast at any meeting at which a quorum is present.

Section 7. Action Without Meeting.

(a) Any action required to be taken at any annual or special meeting of stockholders, or any action that may be taken at any annual or special meeting of stockholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing, setting forth the action so taken, shall be (1) signed and dated by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted and (2) delivered to the Corporation within sixty (60) days of the earliest dated consent by delivery to its registered office in the State of Delaware (in which case delivery shall be by hand or by certified or registered mail, return receipt requested), its principal place of business, or an officer or agent of the Corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Except as otherwise provided by the Certificate of Incorporation, stockholders may act by written consent to elect directors; *provided*, *however*, that, if such consent is less than unanimous, such action by written consent may be in lieu of holding an annual meeting only if all of the directorships to which directors could be elected at an annual meeting held at the effective time of such action are vacant and are filled by such action.

(b) An electronic transmission consenting to an action to be taken and transmitted by a stockholder or proxyholder, or by a person or persons authorized to act for a stockholder or proxyholder, shall be deemed to be written, signed and dated for the purposes of this Section, *provided* that any such electronic transmission sets forth or is delivered with information from which the Corporation can determine (i) that the electronic transmission was

transmitted by the stockholder or proxyholder or by a person or persons authorized to act for the stockholder or proxyholder and (ii) the date on which such stockholder or proxyholder or authorized person or persons transmitted such electronic transmission. The date on which such electronic transmission is transmitted shall be deemed to be the date on which such consent was signed. No consent given by electronic transmission shall be deemed to have been delivered until such consent is reproduced in paper form and until such paper form shall be delivered to the Corporation by delivery to its registered office in the State of Delaware, its principal place of business or an officer or agent of the Corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to the Corporation's registered office shall be made by hand or by certified or registered mail, return receipt requested. Notwithstanding the foregoing limitations on delivery, consents given by electronic transmission may be otherwise delivered to the principal place of business of the Corporation or to an officer or agent of the Corporation having custody of the book in which proceedings of meetings of stockholders are recorded if, to the extent and in the manner provided by resolution of the Board of Directors. Any copy, facsimile or other reliable reproduction of a consent in writing may be substituted or used in lieu of the original writing for any and all purposes for which the original writing could be used, provided that such copy, facsimile or other reproduction shall be a complete reproduction of the entire original writing.

(c) Prompt notice of the taking of the corporate action without a meeting by less than unanimous written consent shall be given to those stockholders who have not consented in writing and who, if the action had been taken at a meeting, would have been entitled to notice of the meeting if the record date for such meeting had been the date that written consents signed by a sufficient number of holders to take the action were delivered to the Corporation.

Section 8. Stock List.

A complete list of stockholders entitled to vote at any meeting of stockholders, arranged in alphabetical order for each class of stock and showing the address of each such stockholder and the number of shares registered in his or her name, shall be open to the examination of any such stockholder, for any purpose germane to the meeting and for a period of at least ten (10) days prior to the meeting, either (a) on a reasonably accessible electronic network, *provided* that the information required to gain access to such list is provided with the notice of the meeting, or (b) during ordinary business hours, at the principal place of business of the Corporation. If the meeting is to be held at a physical location (and not solely by means of remote communication), then the stock list shall be kept at the place of the meeting during the whole time thereof and shall be open to the examination of any such stockholder who is present. If the meeting is to be held solely by means of remote communication, then the list shall also be open to the examination of any stockholder during the whole time of the meeting on a reasonably accessible electronic network, and the information required to gain access to such list shall be provided with the notice of the meeting. Such list shall presumptively determine the identity of the stockholders entitled to vote at the meeting and the number of shares held by each of them.

ARTICLE II
BOARD OF DIRECTORS

Section 1. Number, Election, Tenure and Qualification.

The number of directors that shall constitute the whole Board of Directors shall be eleven (11) directors. The directors shall be elected at any annual or special meeting of the Corporation's stockholders, or upon an action by written consent, called or solicited for such purpose, in accordance with the voting provisions set forth in the Corporation's Certificate of Incorporation and in the Voting Agreement, and each director shall hold office until his or her successor is elected and qualified, or until such director's earlier death, resignation or removal. Directors need not be stockholders.

Section 2. Classes of Directors.

The Board of Directors shall be and is divided into three classes, designated Class I, Class II and Class III. Each class shall consist, as nearly as may be possible, of one-third of the total number of directors constituting the entire Board of Directors. The initial assignment of directors to each of Class I, Class II and Class III shall be determined by resolution of the Board of Directors.

Section 3. Terms of Office.

Each director shall serve for a term ending on the date of the third annual meeting of stockholders following the annual meeting of stockholders at which such director was elected; provided that each director initially assigned to Class I shall serve for a term expiring at the corporation's first annual meeting of stockholders held after the effectiveness of these By-laws; each director initially assigned to Class II shall serve for a term expiring at the corporation's second annual meeting of stockholders held after the effectiveness of these By-laws; and each director initially assigned to Class III shall serve for a term expiring at the corporation's third annual meeting of stockholders held after the effectiveness of these By-laws; provided further, that the term of each director shall continue until the election and qualification of his or her successor and be subject to his or her earlier death, resignation or removal.

Section 4. Removal.

Directors of the corporation may be removed either for cause or without cause by the affirmative vote of the holders of at least a majority of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors

Section 5. Vacancies and Newly Created Directorships.

Vacancies in the office of a director elected by a specified group of stockholders shall be filled in accordance with the provisions set forth in the Corporation's Certificate of Incorporation and, to the extent applicable, the Voting Agreement. Any other vacancies in the office of a director shall be filled by a vote of a majority of the directors then in office, although less than a quorum. Except as provided in the Corporation's Certificate of Incorporation and the Voting Agreement, a director elected to fill a vacancy shall be elected for the unexpired term of such director's

predecessor in office and until a successor is elected and qualified, or until such director's earlier death, resignation or removal. New directorships of the Board of Directors may only be created in accordance with the voting provisions set forth in the Corporation's Certificate of Incorporation and may only be filled in accordance with the provisions set forth in the Corporation's Certificate of Incorporation and the Voting Agreement.

Section 6. Resignation.

Any director may resign at any time upon written notice to the Corporation at its principal place of business or to the President or Secretary. Such resignation shall be effective upon receipt unless it is specified to be effective at some other time or upon the happening of some other event.

Section 7. Regular Meetings.

Regular meetings of the Board of Directors shall be held at such place or places, if any, on such date or dates, and at such time or times as shall have been established by the Board of Directors and publicized among all directors. A written notice of each regular meeting shall not be required.

Section 8. Special Meetings.

Special meetings of the Board of Directors may be called by the Chairman of the Board of Directors, if any, the President, the Treasurer, the Secretary or one or more of the directors then in office and shall be held at such place, if any, on such date, and at such time as they or he or she shall fix. Notice of the place, if any, date, and time of each such special meeting shall be given to each director by whom it is not waived by mailing written notice not less than three (3) days before the meeting or orally or by electronic transmission given not less than twenty-four (24) hours before the meeting. Unless otherwise indicated in the notice thereof, any and all business may be transacted at a special meeting.

Section 9. Quorum.

At any meeting of the Board of Directors, a majority of the total number of members of the Board of Directors shall constitute a quorum for all purposes. If a quorum shall fail to attend any meeting, a majority of those present may adjourn the meeting to another place, date, or time, without further notice or waiver thereof.

Section 10. Action by Consent.

Unless otherwise restricted by the Certificate of Incorporation or these Bylaws, any action required or permitted to be taken at any meeting of the Board of Directors or of any committee thereof, including pursuant to Article X, may be taken without a meeting, if all members of the Board of Directors or committee, as the case may be, consent thereto in writing or by electronic transmission, and the writing or written consents or electronic transmissions are filed with the minutes of proceedings of the Board of Directors or committee. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form.

Section 11. Participation in Meetings By Conference Telephone.

Members of the Board of Directors, or of any committee thereof, may participate in a meeting of the Board of Directors or any such committee by way of conference telephone or similar communications equipment by means of which all persons participating in the meeting can hear each other and such participation shall constitute presence in person at such meeting.

Section 12. Conduct of Business.

At any meeting of the Board of Directors, or of any committee thereof, business shall be transacted in such order and manner as the Board of Directors, or such committee, may from time to time determine, and all matters shall be determined by the vote of a majority of the directors present at such meeting, except as otherwise provided herein or required by law.

Section 13. Powers.

The Board of Directors may, except as otherwise required by law or the Certificate of Incorporation, exercise all such powers and do all such acts and things as may be exercised or done by the Corporation, including, without limiting the generality of the foregoing, the qualified power:

(a) to declare dividends from time to time in accordance with law and the Certificate of Incorporation;

(b) to purchase or otherwise acquire any property, rights or privileges on such terms as it shall determine;

(c) to authorize the creation, making and issuance, in such form as it may determine, of written obligations of every kind, negotiable or non-negotiable, secured or unsecured, to borrow funds and guarantee obligations, and to do all things necessary in connection therewith;

(d) to remove any officer of the Corporation with or without cause, and from time to time to devolve the powers and duties of any officer upon any other person for the time being;

(e) to confer upon any officer of the Corporation the power to appoint, remove and suspend subordinate officers, employees and agents;

(f) to adopt from time to time such stock, option, stock purchase, bonus or other compensation plans of directors, officers, employees and agents of the Corporation and its subsidiaries as it may determine;

(g) to adopt from time to time such insurance, retirement, and other benefit plans for directors, officers, employees and agents of the Corporation and its subsidiaries as it may determine; and

(h) to adopt from time to time regulations, not inconsistent with these Bylaws, for the management of the Corporation’s business and affairs.

Section 14. Compensation of Directors.

Directors, as such, may receive, pursuant to a resolution of the Board of Directors, fixed fees and other compensation for their services as directors, including, without limitation, their services as members of committees of the Board of Directors.

Section 15. Chairman of the Board.

The Chairman of the Board, if any, and any Vice Chairman of the Board appointed to act in the absence of the Chairman, if any, shall be elected by and from the Board of Directors. The Chairman of the Board, if any, shall preside at all meetings of the Board of Directors and stockholders at which he or she is present and shall have such authority and perform such duties as may be prescribed by these Bylaws or from time to time be determined by the Board of Directors.

ARTICLE III
COMMITTEES

Section 1. Committees of the Board of Directors.

The Board of Directors, by a vote of a majority of the Board of Directors, may from time to time designate committees of the Board of Directors, with such lawfully delegable powers and duties as it thereby confers, to serve at the pleasure of the Board of Directors and shall, for those committees and any others provided for herein, elect a director or directors, subject to the rights of any preferred stockholder pursuant to the Voting Agreement, to serve as the member or members, designating, if it desires other directors as alternate members who may replace any absent or disqualified member at any meeting of the committee. Any such committee, to the extent provided in the resolution of the Board of Directors, shall have and may exercise all the powers and authority of the Board of Directors in the management of the business and affairs of the Corporation, and may authorize the seal of the Corporation to be affixed to all papers which may require it; but no such committee shall have the power or authority in reference to amending the Certificate of Incorporation, adopting an agreement of merger or consolidation, recommending to the stockholders the sale, lease, or exchange of all or substantially all of the Corporation’s property and assets, recommending to the stockholders a dissolution of the Corporation or a revocation of a dissolution, or amending the Bylaws of the Corporation. Any committee so designated may exercise the power and authority of the Board of Directors to declare a dividend, to authorize the issuance of stock or to adopt a certificate of ownership and merger pursuant to Section 253 of the Delaware General Corporation Law if the resolution that designates the committee or a supplemental resolution of the Board of Directors shall so provide. In the absence or disqualification of any member of any committee and any alternate member in his or her place, the member or members of the committee present at the meeting and not disqualified from voting, whether or not he or she or they constitute a quorum, may by unanimous vote appoint another member of the Board of Directors to act at the meeting in the place of the absent or disqualified member subject to the rights of any preferred stockholder pursuant to the Voting Agreement.

Section 2. Conduct of Business.

Each Committee may determine the procedural rules for meeting and conducting its business and shall act in accordance therewith, except as otherwise provided herein or required by law. Adequate provision shall be made for notice to members of all meetings; one-third (1/3) of the members shall constitute a quorum; and all matters shall be determined by a majority vote of the members present. Action may be taken by any committee without a meeting if all members thereof consent thereto in writing or by electronic transmission, and the written consents or electronic transmissions are filed with the minutes of the proceedings of such committee.

ARTICLE IV
OFFICERS

Section 1. Enumeration.

The officers of the Corporation shall be the President, the Treasurer, the Secretary and such other officers as the Board of Directors or the Chairman of the Board may determine, including, but not limited to, one or more Vice Presidents, Assistant Treasurers and Assistant Secretaries.

Section 2. Election and Compensation.

Officers shall be elected by the Board of Directors at its first meeting following every annual meeting of stockholders. The compensation of the officers elected by the Board of Directors shall be fixed from time to time by the Board of Directors or a committee thereof.

Section 3. Qualification.

No officer need be a stockholder. Two or more offices may be held by any one person.

Section 4. Tenure and Removal.

Each officer shall hold office until the first meeting of the Board of Directors following the next annual meeting of the stockholders and until his or her successor is elected or appointed and qualified, or until he or she dies, resigns, is removed or becomes disqualified, unless a shorter term is specified in the vote electing or appointing said officer. Any officer may resign by giving written notice of his or her resignation to the Chairman of the Board, if any, the President, or the Secretary, or to the Board of Directors at a meeting of the Board of Directors, and such resignation shall become effective at the time specified therein. Any officer may be removed from office with or without cause by vote of a majority of the directors. Except as the Board of Directors may otherwise determine, no officer who resigns or is removed shall have any right to any compensation as an officer for any period following such officer's removal, whether such officer's compensation be by the month or by the year or otherwise, unless such compensation is expressly provided for in a duly authorized written agreement with the Corporation.

Section 5. Vacancies.

The Board of Directors may fill any vacancy occurring in any office for any reason and may, in its discretion, leave unfilled for such period as it may determine any offices other than those of

President, Treasurer and Secretary. Each such successor shall hold office for the unexpired term of such officer's predecessor and until a predecessor is elected and qualified, or until such officer's earlier death, resignation or removal.

Section 6. President.

The President shall, subject to the control and direction of the Board of Directors, have and perform such powers and duties that are commonly incident to the office of president or that are delegated to such officer by the Board of Directors.

Section 7. Vice Presidents.

The Vice Presidents, if any, in the order of their election, or in such other order as the Board of Directors may determine, shall have and perform the powers and duties of the President (or such of the powers and duties as the Board of Directors may determine) whenever the President is absent or unable to act. The Vice Presidents, if any, shall also have such other powers and duties as may from time to time be delegated by the Board of Directors.

Section 8. Treasurer and Assistant Treasurers.

The Treasurer shall, subject to the control and direction of the Board of Directors, have and perform such powers and duties as may be prescribed in these Bylaws or be delegated from time to time by the Board of Directors. All property of the Corporation in the custody of the Treasurer shall be subject at all times to the inspection and control of the Board of Directors. Unless otherwise voted by the Board of Directors, each Assistant Treasurer, if any, shall have and perform the powers and duties of the Treasurer whenever the Treasurer is absent or unable to act, and may at any time exercise such of the powers of the Treasurer, and such other powers and duties, as may from to time be determined by the Board of Directors.

Section 9. Secretary and Assistant Secretaries.

The Board of Directors shall appoint a Secretary and, in his or her absence, an Assistant Secretary. The Secretary or, in his or her absence, any Assistant Secretary, shall attend all meetings of the directors and shall record all votes of the Board of Directors and minutes of the proceedings at such meetings. The Secretary or, in his or her absence, any Assistant Secretary, shall notify the directors of their meetings, and shall have and perform such other powers and duties as may from time to time be delegated by the Board of Directors. If the Secretary or an Assistant Secretary is elected but is absent from any meeting of directors, a temporary secretary may be appointed by the directors at the meeting.

Section 10. Bond.

If required by the Board of Directors, any officer shall give the Corporation a bond in such sum and with such surety or sureties and upon such terms and conditions as shall be satisfactory to the Board of Directors, including without limitation a bond for the faithful performance of the duties of his office and for the

restoration to the Corporation of all books, papers, vouchers, money and other property of whatever kind in his or her possession or under his or her control and belonging to the Corporation. The premiums for such bond shall be paid by the Corporation.

Section 11. Action with Respect to Securities of Other Corporations.

Unless otherwise directed by the Board of Directors, the President, the Treasurer or any officer of the Corporation authorized by the President shall have power to vote and otherwise act on behalf of the Corporation, in person or by proxy, at any meeting of stockholders of, or with respect to any action of stockholders of any other corporation in which this Corporation may hold securities and otherwise to exercise any and all rights and powers which this Corporation may possess by reason of its ownership of securities in such other corporation.

Section 12. Delegation of Authority.

The Board of Directors may from time to time delegate the powers or duties of an officer to any other officer or agent, notwithstanding any provision hereof.

Section 13. Evidence of Authority.

A certificate by the Secretary, or an Assistant Secretary, or a temporary Secretary, as to any action taken by the stockholders, directors, a committee or any officer or representative of the corporation shall as to all persons who rely on the certificate in good faith be conclusive evidence of such action.

ARTICLE V
STOCK

Section 1. Stock Certificates; Uncertificated Shares.

The shares of the Corporation shall be represented by certificates, provided that the Board of Directors may provide by resolution or resolutions that some or all of any or all classes or series of the Corporation's stock shall be uncertificated shares. Every holder of stock of the Corporation represented by certificates shall be entitled to have a certificate, in such form as may be prescribed by law and by the Board of Directors, representing the number of shares held by such holder registered in certificate form. Each such certificate shall be signed in a manner that complies with Section 158 of the General Corporation Law of the State of Delaware.

Each certificate for shares of stock which are subject to any restriction on transfer pursuant to the Certificate of Incorporation, these Bylaws, applicable securities laws or any agreement among any number of stockholders or among such holders and the Corporation shall have conspicuously noted on the face or back of the certificate either the full text of the restriction or a statement of the existence of such restriction.

If the Corporation shall be authorized to issue more than one class of stock or more than one series of any class, the powers, designations, preferences and relative participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights shall be set forth in full or summarized on the face or back of each certificate representing shares of such class or series of stock, provided that in lieu of the foregoing requirements there may be set forth on the face or back of each certificate representing shares of such class or series of stock a statement that the Corporation will furnish

without charge to each stockholder who so requests a copy of the full text of the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights.

Within a reasonable time after the issuance or transfer of uncertificated shares, the Corporation shall send to the registered owner thereof a written notice containing the information required to be set forth or stated on certificates pursuant to Sections 151, 202(a) or 218(a) of the General Corporation Law of the State of Delaware or, with respect to Section 151 of General Corporation Law of the State of Delaware, a statement that the Corporation will furnish without charge to each stockholder who so requests the powers, designations, preferences and relative participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights.

Section 2. Transfers of Stock.

Transfers of stock shall be made only upon the transfer books of the Corporation kept at an office of the Corporation or by transfer agents designated to transfer shares of the stock of the Corporation. Except where a certificate is issued in accordance with Section 4 of this Article of these Bylaws, shares of stock represented by certificates shall be transferred only on the books of the Corporation by the surrender to the Corporation or its transfer agent of the certificate representing such shares properly endorsed or accompanied by a written assignment or power of attorney properly executed, and with such proof of authority or the authenticity of signature as the Corporation or its transfer agent may reasonably require. Except as may be otherwise required by law, by the Certificate of Incorporation or by these Bylaws, the Corporation shall be entitled to treat the record holder of stock as shown on its books as the owner of such stock for all purposes, including the payment of dividends and the right to vote with respect to such stock, regardless of any transfer, pledge or other disposition of such stock until the shares have been transferred on the books of the Corporation in accordance with the requirements of these Bylaws.

Section 3. Record Date.

In order that the Corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders, or to receive payment of any dividend or other distribution or allotment of any rights or to exercise any rights in respect of any change, conversion or exchange of stock or for the purpose of any other lawful action, the Board of Directors may fix a record date, subject to the notice provisions of the Certificate of Incorporation, which record date shall not precede the date on which the resolution fixing the record date is adopted and which record date shall not be more than sixty (60) nor less than ten (10) days before the date of any meeting of stockholders, nor more than ten (10) days after the date of adoption of a record date for a consent without a meeting, nor more than sixty (60) days prior to the time for such other action hereinbefore described. If no record date is fixed by the Board of Directors, the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given or, if notice is waived, at the close of business on the day next preceding the day on which the meeting is held. If no record date is fixed by the Board of Directors, the record date for determining stockholders entitled to express consent to corporate action without a meeting, when no prior action by the

Board of Directors is necessary, shall be the day on which the first consent is properly delivered to the Corporation. If no record date is fixed by the Board of Directors, the record date for determining stockholders entitled to receive payment of any dividend or other distribution or allotment of rights or to exercise any rights of change, conversion or exchange of stock or for any other purpose, the record date shall be at the close of business on the day on which the Board of Directors adopts a resolution relating thereto.

A determination of stockholders of record entitled to notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting; provided, however, that the Board of Directors may fix a new record date for the adjourned meeting.

Section 4. Lost, Stolen or Destroyed Certificates.

In the event of the loss, theft or destruction of any certificate of stock, another may be issued in its place pursuant to such regulations as the Board of Directors may establish concerning proof of such loss, theft or destruction and concerning the giving of a satisfactory bond or bonds of indemnity.

Section 5. Regulations.

The issue, transfer, conversion and registration of certificates of stock shall be governed by such other regulations as the Board of Directors may establish.

ARTICLE VI
WAIVER OF NOTICE

Section 1. Waiver of Notice.

A written waiver of any notice, signed by a stockholder, director, officer, employee or agent, whether before or after the time of the event for which notice is to be given, shall be deemed equivalent to the notice required to be given to such stockholder, director, officer, employee or agent. Neither the business nor the purpose of any meeting need be specified in such a waiver. Attendance of a director or stockholder at a meeting without protesting prior thereto or at its commencement the lack of notice shall also constitute a waiver of notice by such director or stockholder.

ARTICLE VII
INDEMNIFICATION

Section 1. Actions other than by or in the Right of the Corporation.

The Corporation shall indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than action by or in the right of the Corporation) by reason of the fact that he or she is or was a director, officer, employee or agent of the Corporation, or is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and

reasonably incurred by him or her in connection with such action, suit or proceeding if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the Corporation, and, with respect to any criminal action or proceedings, had no reasonable cause to believe his or her conduct was unlawful. The termination of any action, suit or proceeding by judgment, order, settlement, conviction, or upon a plea of *nolo contendere* or its equivalent, shall not, of itself, create a presumption that the person did not act in good faith and in a manner that he or she reasonably believed to be in or not opposed to the best interests of the Corporation, and, with respect to any criminal action or proceeding, had reasonable cause to believe that his or her conduct was unlawful.

Section 2. Actions by or in the Right of the Corporation.

The Corporation shall indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the Corporation to procure a judgment in its favor by reason of the fact that he or she is or was a director, officer, employee or agent of the Corporation, or is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust, or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by him or her in connection with the defense or settlement of such action or suit if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the Corporation and except that no indemnification shall be made in respect of any claim, issue or matter as to which such person shall have been adjudged to be liable to the Corporation unless and only to the extent that the Court of Chancery of the State of Delaware or the court in which such action or suit was brought shall determine upon application that, despite the adjudication of liability but in view of all the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses that the Court of Chancery of the State of Delaware or such other court shall deem proper.

Section 3. Success on the Merits.

To the extent that a present or former director or officer of the Corporation has been successful on the merits or otherwise in defense of any action, suit or proceeding referred to Section 1 or Section 2 of this Article, or in defense of any claim, issue or matter therein, he or she shall be indemnified against expenses (including attorneys' fees) actually and reasonably incurred by him or her in connection therewith.

Section 4. Specific Authorization.

Any indemnification under Section 1 or Section 2 of this Article (unless ordered by a court) shall be made by the Corporation only as authorized in the specific case upon a determination that indemnification of any person described in said Sections is proper in the circumstances because he or she has met the applicable standard of conduct set forth in said Sections. Such determination shall be made (1) by a majority vote of directors who were not parties to such action, suit or proceeding, even though less than a quorum, or (2) by a committee of such directors designated by majority vote of such directors, even though less than a

quorum, (3) if there are no such directors, or if such directors so direct, by independent legal counsel in a written opinion, or (4) by the stockholders of the Corporation.

Section 5. Advance Payment.

Expenses incurred by an officer or director in defending any civil, criminal, administrative, or investigative action, suit or proceeding may be paid by the Corporation in advance of the final disposition of such action, suit or proceeding upon receipt of an undertaking by or on behalf of such officer or director to repay such amount if it shall ultimately be determined that he or she is not entitled to indemnification by the Corporation as authorized in this Article.

Section 6. Non-Exclusivity.

The indemnification and advancement of expenses provided by, or granted pursuant to, the other Sections of this Article shall not be deemed exclusive of any other rights to which those seeking indemnification or advancement of expenses may be entitled under any bylaw, agreement, vote of stockholders or disinterested directors or otherwise, both as to action in such person's official capacity and as to action in another capacity while holding such office.

Section 7. Insurance.

The Board of Directors may authorize, by a vote of the majority of the full Board of Directors, the Corporation to purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of the Corporation, or is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against him or her and incurred by him or her in any such capacity, or arising out of his or her status as such, whether or not the Corporation would have the power to indemnify him or her against such liability under the provisions of this Article.

Section 8. Continuation of Indemnification and Advancement of Expenses.

The indemnification and advancement of expenses provided by, or granted pursuant to, this Article shall, unless otherwise provided when authorized or ratified, continue as to a person who has ceased to be a director, officer, employee or agent and shall inure to the benefit of the heirs, executors and administrators of such a person.

Section 9. Intent of Article.

The intent of this Article is to provide for indemnification and advancement of expenses to the fullest extent permitted by Section 145 of the General Corporation Law of Delaware. To the extent that such Section or any successor section may be amended or supplemented from time to time, this Article shall be amended automatically and construed so as to permit indemnification and advancement of expenses to the fullest extent from time to time permitted by law.

ARTICLE VIII
CERTAIN TRANSACTIONS

Section 1. Transactions with Interested Parties.

No contract or transaction between the Corporation and one or more of its directors or officers, or between the Corporation and any other corporation, partnership, association, or other organization in which one or more of its directors or officers are directors or officers, or have a financial interest, shall be void or voidable solely for this reason, or solely because the director or officer is present at or participates in the meeting of the Board of Directors or committee thereof which authorizes the contract or transaction, or solely because the votes of such director or officer are counted for such purpose, if:

(a) The material facts as to his or her relationship or interest and as to the contract or transaction are disclosed or are known to the Board of Directors or the committee, and the Board of Directors or committee in good faith authorizes the contract or transaction by the affirmative votes of a majority of the disinterested directors, even though the disinterested directors be less than a quorum; or

(b) The material facts as to his or her relationship or interest and as to the contract or transaction are disclosed or are known to the stockholders entitled to vote thereon, and the contract or transaction is specifically approved in good faith by vote of the stockholders; or

(c) The contract or transaction is fair as to the Corporation as of the time it is authorized, approved or ratified, by the Board of Directors, a committee thereof, or the stockholders.

Section 2. Quorum.

Common or interested directors may be counted in determining the presence of a quorum at a meeting of the Board of Directors or of a committee that authorizes the contract or transaction.

ARTICLE IX
MISCELLANEOUS

Section 1. Facsimile Signatures

In addition to the provisions for use of facsimile signatures elsewhere specifically authorized in these Bylaws, facsimile signatures of any officer or officers of the Corporation may be used whenever and as authorized by the Board of Directors or a committee thereof.

Section 2. Corporate Seal.

The Board of Directors may provide a suitable seal, containing the name of the Corporation, which shall be in the charge of the Secretary. If and when so directed by the Board of Directors or a committee thereof, duplicates of the seal may be kept and used by the Treasurer or by an Assistant Secretary or Assistant Treasurer.

Section 3. Reliance upon Books, Reports and Records.

Each director, each member of any committee designated by the Board of Directors, and each officer of the Corporation shall, in the performance of his or her duties, be fully protected in relying in good faith upon the books of account or other records of the Corporation and upon such information, opinions, reports or statements presented to the Corporation by any of its officers or employees, or committees of the Board of Directors so designated, or by any other person as to matters which such director or committee member reasonably believes are within such other person's professional or expert competence and who has been selected with reasonable care by or on behalf of the Corporation.

Section 4. Fiscal Year

Except as otherwise determined by the Board of Directors from time to time, the fiscal year of the Corporation shall end on the last day of December of each year.

Section 5. Time Periods.

In applying any provision of these Bylaws which requires that an act be done or not be done on a specified number of days prior to an event or that an act be done during a period of a specified number of days prior to an event, calendar days shall be used, the day of the doing of the act shall be excluded, and the day of the event shall be included.

Section 6. Severability.

If any word, clause or provision of these Bylaws or any award made hereunder shall for any reason be determined to be invalid, the provisions hereof shall not otherwise be affected thereby but shall remain in full force and effect.

Section 7. Interpretation.

Subject to Article XI of these Bylaws, the Board of Directors shall have the power to interpret all of the terms and provisions of these Bylaws, which interpretation shall be conclusive.

ARTICLE X
AMENDMENTS

These Bylaws may be amended, added to, rescinded or repealed by the stockholders or by the Board of Directors, when such power is conferred upon the Board of Directors by the Certificate of Incorporation, at any meeting of the stockholders or of the Board of Directors, respectively, *provided* notice of the proposed change was given in the notice of the meeting or, in the case of a meeting of the Board of Directors, a notice given not less than two (2) days prior to the meeting.

ARTICLE XI
CONFLICTS

In the event of a conflict or inconsistency between the provisions of these Bylaws and the provisions of the Second Amended and Restated Investors' Rights Agreement, made as of March

7, 2013 by and among the Corporation and the other signatories thereto, and/or the Voting Agreement (together, as the same may be amended or restated from time to time, the "Agreements"), the provisions of the Agreements shall control.

Exhibit 4.2

EXECUTION COPY

PTC THERAPEUTICS, INC.
SECOND AMENDED AND RESTATED
INVESTORS' RIGHTS AGREEMENT

Dated as of March 7, 2013

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SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

THIS SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT, is entered into as of March 7, 2013 (this "**Agreement**"), among PTC THERAPEUTICS, INC., a Delaware corporation (the "**Company**"), and each of the investors listed on Schedule A and Schedule B hereto (each of which is referred to in this Agreement as an "**Investor**").

RECITALS

WHEREAS, Concurrently with the execution of this Agreement, the Company and certain of the Investors are entering into a Subscription Agreement (the "**Subscription Agreement**") providing for the sale and issuance of shares of the Series Four Senior Preferred Stock to such Investors; and

WHEREAS, The Company and certain of its securityholders are parties to the Amended and Restated Investors' Rights Agreement, dated as of May 29, 2012 (the "**Prior IRA**"), and desire to amend and restate the Prior IRA in its entirety and accept the rights created pursuant to this Agreement in lieu of the rights granted to them under the Prior IRA;

NOW, THEREFORE, the parties to this Agreement further agree as follows:

1. Definitions. For purposes of this Agreement:

1.1. "**Affiliate**" means, with respect to any specified Person, any other Person who, directly or indirectly, controls, is controlled by, or is under common control with such Person, and such term shall include, without limitation, any general partner, managing member, officer or director of such Person or any venture capital or other investment fund now or hereafter existing that is controlled by one or more general partners or managing members of, or shares the same management company or investment adviser with, such Person (whether or not such other Person would be an "affiliate" of such Person under the Securities Act or the Exchange Act).

1.2. "**Common Stock**" means shares of the Company's common stock, $0.001 par value per share.

1.3. "**Damages**" means any loss, damage, claim or liability (joint or several) to which a party hereto may become subject under the Securities Act, the Exchange Act or other federal or state law, insofar as such loss, damage, claim or liability (or any action in respect thereof) arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material fact contained in any registration statement of the Company, including any preliminary prospectus or final prospectus contained therein or any amendments or supplements thereto; (ii) an omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading; or (iii) any violation or alleged violation by the indemnifying party (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated thereunder.

1.4. "**Designated Investors**" means each of Delphi Ventures V, L.P., Novo A/S, The Column Group, L.P. and Section Six Partners, L.P.

1.5. "**Derivative Securities**" means any securities or rights convertible into, or exercisable or exchangeable for (in each case, directly or indirectly)**,** Common Stock, including options and warrants.

1.6. "**Exchange Act**" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

1.7. "**Excluded Registration**" means (i) a registration relating to the sale of securities to employees of the Company or a subsidiary pursuant to a stock option, stock purchase, or similar plan; (ii) a registration relating to an SEC Rule 145 transaction; (iii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities; or (iv) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

1.8. "**Form S-1**" means such form under the Securities Act as in effect on the date hereof or any successor registration form under the Securities Act subsequently adopted by the SEC.

1.9. "**Form S-3**" means such form under the Securities Act as in effect on the date hereof or any registration form under the Securities Act subsequently adopted by the SEC that permits incorporation of substantial information by reference to other documents filed by the Company with the SEC.

1.10. "**GAAP**" means generally accepted accounting principles in the United States.

1.11. "**Holder**" means any holder of Registrable Securities who is a party to this Agreement.

1.12. "**Immediate Family Member**" means a child, stepchild, grandchild, parent, stepparent, grandparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, or domestic partner of a natural person referred to herein.

1.13. "**Initiating Holders**" means, collectively, Holders who properly initiate a registration request under this Agreement.

1.14. "**IPO**" means the Company's first underwritten public offering of its Common Stock under the Securities Act.

1.15. "**Key Employee**" means any executive-level employee (including division director and vice president-level positions) as well as any employee who, either alone or in concert with others, develops, invents, programs, or designs any Company Intellectual Property (as defined in the Subscription Agreement).

1.16. "**Major Investor**" means any Investor that, individually or together with such Investor's Affiliates, holds Series Four Senior Preferred Stock and/or Series Five Junior Preferred Stock, in the aggregate, convertible into at least 3,696 shares of Common Stock (as such number is adjusted for any stock split, stock dividend, combination, or other recapitalization or reclassification effected after the date hereof).

1.17. "**Minimum Threshold**" means (a) for Delphi Ventures V, L.P., Preferred Stock convertible into at least 143,365 shares of Common Stock; (b) for Novo A/S, Preferred Stock convertible into at least 89,604 shares of Common Stock; (c) for The Column Group, L.P., Preferred Stock convertible into at least 79,973 shares of Common Stock; and (d) for Section Six Partners, L.P., Preferred Stock convertible into at least 114,605 shares of Common Stock.

1.18. "**New Securities**" means, collectively, equity securities of the Company, whether or not currently authorized, as well as rights, options, or warrants to purchase such equity securities, or securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for such equity securities.

1.19. "**Person**" means any individual, corporation, partnership, trust, limited liability company, association or other entity.

1.20. "**Preferred Stock**" means, collectively, shares of the Company's Series Four Senior Preferred Stock and Series Five Junior Preferred Stock.

1.21. "**Registrable Securities**" means (i) the Common Stock issuable or issued upon conversion of the Preferred Stock; (ii) any Common Stock, or any Common Stock issued or issuable (directly or indirectly) upon conversion and/or exercise of any other securities of the Company, acquired by the Investors after the date hereof; and (iii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clauses (i) and (ii) above; excluding in all cases, however, any Registrable Securities sold by a Person in a transaction in which the applicable rights under this Agreement are not assigned pursuant to Subsection 6.1, and excluding for purposes of Section 2 any shares for which registration rights have terminated pursuant to Subsection 2.13 of this Agreement.

1.22. "**Registrable Securities then outstanding**" means the number of shares determined by adding the number of shares of outstanding Common Stock that are Registrable Securities and the number of shares of Common Stock issuable (directly or indirectly) pursuant to then exercisable and/or convertible securities that are Registrable Securities.

1.23. "**Restricted Securities**" means the securities of the Company required to bear the legend set forth in Subsection 2.12(b) hereof or for which the Company has committed to providing the notation set forth in Subsection 2.12(c) hereof in any notice containing information required to be set forth pursuant to Section 202(a) of the General Corporation Law of the State of Delaware.

1.24. "**SEC**" means the Securities and Exchange Commission.

1.25. "**SEC Rule 144**" means Rule 144 promulgated by the SEC under the Securities Act.

1.26. "**SEC Rule 145**" means Rule 145 promulgated by the SEC under the Securities Act.

1.27. "**Securities Act**" means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

1.28. "**Selling Expenses**" means all underwriting discounts, selling commissions, and stock transfer taxes applicable to the sale of Registrable Securities, and fees and disbursements of counsel for any Holder, except for the fees and disbursements of the Selling Holder Counsel borne and paid by the Company as provided in Subsection 2.6.

1.29. "**Series Five Junior Preferred Stock**" means shares of the Company's Series Five Junior Preferred Stock, $0.001 par value per share.

1.30. "**Series Four Senior Preferred Stock**" means shares of the Company's Series Four Senior Preferred Stock, $0.001 par value per share.

2. Registration Rights. The Company covenants and agrees as follows:

2.1. Demand Registration.

(a) Form S-1 Demand. If at any time after one hundred eighty (180) days after the effective date of the registration statement for the IPO, the Company receives a request from Holders of at least twenty percent (20%) of the Registrable Securities then outstanding that the Company file a Form S-1 registration statement with respect to at least twenty percent (20%) of the Registrable Securities then outstanding, the anticipated aggregate offering proceeds, net of Selling Expenses, of which would exceed $10 million, then the Company shall (i) within ten (10) days after the date such request is given, give notice thereof (the "**Demand Notice**") to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within sixty (60) days after the date such request is given by the Initiating Holders, file a Form S-1 registration statement under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Subsection 2.1(c) and Subsection 2.3.

(b) Form S-3 Demand. If at any time when it is eligible to use a Form S-3 registration statement, the Company receives a request from Holders of Registrable Securities that the Company file a Form S-3 registration statement with respect to outstanding Registrable Securities of such Holders having an anticipated aggregate offering price, net of Selling Expenses, of at least $5 million, then the Company shall (i) within ten (10) days after the date such request is given, give a Demand Notice to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within forty-five (45) days after the date such request is given by the Initiating Holders, file a Form S-3 registration statement under the Securities Act covering all Registrable Securities requested to be included in such registration by any other

Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Subsection 2.1(c) and Subsection 2.3.

(c) Notwithstanding the foregoing obligations, if the Company furnishes to Holders requesting a registration pursuant to this Subsection 2.1 a certificate signed by the Company's chief executive officer stating that in the good faith judgment of the Company's Board of Directors it would be seriously detrimental to the Company and its stockholders for a registration statement to be filed in the near future, then the Company shall have the right to defer taking action with respect to such filing, and any time periods with respect to filing or effectiveness thereof shall be tolled correspondingly, for a period of not more than ninety (90) days after the request of the Initiating Holders is given; provided, however, that the Company may not invoke this right more than once in any twelve (12) month period; provided, further, however, that it shall be considered seriously detrimental to the Company and its stockholders for a registration statement to be filed in the near future if the Company shall be preparing in good faith to commence any offering of securities at the time any such request by the Initiating Holders is made.

(d) The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Subsection 2.1(a) (i) during the period that is sixty (60) days before the Company's good faith estimate of the date of filing of, and ending on a date that is one hundred eighty (180) days after the effective date of, a Company-initiated registration, provided, that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; (ii) after the Company has effected two (2) registrations pursuant to Subsection 2.1(a); or (iii) if the Initiating Holders propose to dispose of shares of Registrable Securities that may be immediately registered on Form S-3 pursuant to a request made pursuant to Subsection 2.1(b). The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Subsection 2.1(b) (i) during the period that is thirty (30) days before the Company's good faith estimate of the date of filing of, and ending on a date that is ninety (90) days after the effective date of, a Company-initiated registration, provided, that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; or (ii) if the Company has effected four prior (4) registrations pursuant to Subsection 2.1(b). A registration shall not be counted as "effected" for purposes of this Subsection 2.1(d) until such time as the applicable registration statement has been declared effective by the SEC, unless the Initiating Holders withdraw their request for such registration, elect not to pay the registration expenses therefor, and forfeit their right to one demand registration statement pursuant to Subsection 2.6, in which case such withdrawn registration statement shall be counted as "effected" for purposes of this Subsection 2.1(d).

2.2. Company Registration. If the Company proposes to register (including, for this purpose, a registration effected by the Company for stockholders other than the Holders) any of its Common Stock under the Securities Act in connection with the public offering of such securities solely for cash (other than in an Excluded Registration), the Company shall, at such time, promptly give each Holder notice of such registration. Upon the request of each Holder given within twenty (20) days after such notice is given by the Company, the Company shall, subject to the provisions of Subsection 2.3, cause to be registered all of the Registrable Securities

that each such Holder has requested to be included in such registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Subsection 2.2 before the effective date of such registration, whether or not any Holder has elected to include Registrable Securities in such registration. The expenses (other than Selling Expenses) of such withdrawn registration shall be borne by the Company in accordance with Subsection 2.6.

2.3. Underwriting Requirements.

(a) If, pursuant to Subsection 2.1, the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to Subsection 2.1, and the Company shall include such information in the Demand Notice. The underwriter(s) will be selected by the Company and shall be reasonably acceptable to a majority in interest of the Initiating Holders. In such event, the right of any Holder to include such Holder's Registrable Securities in such registration shall be conditioned upon such Holder's participation in such underwriting and the inclusion of such Holder's Registrable Securities in the underwriting to the extent provided herein. All Holders proposing to distribute their securities through such underwriting shall (together with the Company as provided in Subsection 2.4(e)) enter into an underwriting agreement in customary form with the underwriter(s) selected for such underwriting. Notwithstanding any other provision of this Subsection 2.3, if the managing underwriter(s) advise(s) the Company and the Initiating Holders in writing that marketing factors require a limitation on the number of shares to be underwritten, then the Initiating Holders shall so advise all Holders of Registrable Securities that otherwise would be underwritten pursuant hereto, and the number of Registrable Securities that may be included in the underwriting shall be allocated among such Holders of Registrable Securities, including the Initiating Holders, in proportion (as nearly as practicable) to the number of Registrable Securities owned by each Holder or in such other proportion as shall mutually be agreed to by all such selling Holders; provided, however, that the number of Registrable Securities held by the Holders to be included in such underwriting shall not be reduced unless all other securities are first entirely excluded from the underwriting. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest 100 shares.

(b) In connection with any offering involving an underwriting of shares of the Company's capital stock pursuant to Subsection 2.2, the Company shall not be required to include any of the Holders' Registrable Securities in such underwriting unless the Holders accept the terms of the underwriting as agreed upon between the Company and its underwriters, and then only in such quantity as the underwriters in their sole discretion determine will not jeopardize the success of the offering by the Company. If the total number of securities, including Registrable Securities, requested by stockholders to be included in such offering exceeds the number of securities to be sold (other than by the Company) that the underwriters in their reasonable discretion determine is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Registrable Securities, which the underwriters and the Company in their sole discretion determine will not jeopardize the success of the offering. If the underwriters determine that less than all of the Registrable Securities requested to be registered can be included in such offering, then the Registrable Securities that are included in such offering shall be allocated among the

selling Holders in proportion (as nearly as practicable to) the number of Registrable Securities owned by each selling Holder or in such other proportions as shall mutually be agreed to by all such selling Holders. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest 100 shares. Notwithstanding the foregoing, in no event shall (i) the number of Registrable Securities included in the offering be reduced unless all other securities (other than securities to be sold by the Company) are first entirely excluded from the offering, or (ii) the number of Registrable Securities included in the offering be reduced below twenty percent (20%) of the total number of securities included in such offering, unless such offering is the IPO, in which case the selling Holders may be excluded further if the underwriters make the determination described

above and no other stockholder's securities are included in such offering. For purposes of the provision in this Subsection 2.3(b) concerning apportionment, for any selling Holder that is a partnership, limited liability company, or corporation, the partners, members, retired partners, retired members, stockholders, and Affiliates of such Holder, or the estates and Immediate Family Members of any such partners, retired partners, members, and retired members and any trusts for the benefit of any of the foregoing Persons, shall be deemed to be a single "selling Holder", and any pro rata reduction with respect to such "selling Holder" shall be based upon the aggregate number of Registrable Securities owned by all Persons included in such "selling Holder", as defined in this sentence.

(c) For purposes of Subsection 2.1, a registration shall not be counted as "effected" if, as a result of an exercise of the underwriter's cutback provisions in Subsection 2.3(a), fewer than fifty percent (50%) of the total number of Registrable Securities that Holders have requested to be included in such registration statement are actually included.

2.4. Obligations of the Company. Whenever required under this Section 2 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and use its commercially reasonable efforts to cause such registration statement to become effective and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to one hundred twenty (120) days or, if earlier, until the distribution contemplated in the registration statement has been completed; provided, however, that such one hundred twenty (120) day period shall be extended for a period of time equal to the period the Holder refrains, at the request of an underwriter of Common Stock (or other securities) of the Company, from selling any securities included in such registration;

(b) prepare and file with the SEC such amendments and supplements to such registration statement, and the prospectus used in connection with such registration statement, as may be necessary to comply with the Securities Act in order to enable the disposition of all securities covered by such registration statement;

(c) furnish to the selling Holders such numbers of copies of a prospectus, including a preliminary prospectus, as required by the Securities Act, and such other documents

as the Holders may reasonably request in order to facilitate their disposition of their Registrable Securities;

(d) use its commercially reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or blue-sky laws of such jurisdictions as shall be reasonably requested by the selling Holders; provided that the Company shall not be required to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, unless the Company is already subject to service in such jurisdiction and except as may be required by the Securities Act;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the underwriter(s) of such offering;

(f) use its commercially reasonable efforts to cause all such Registrable Securities covered by such registration statement to be listed on a national securities exchange or trading system and each securities exchange and trading system (if any) on which similar securities issued by the Company are then listed;

(g) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and provide a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration;

(h) promptly make available for inspection by the selling Holders, any managing underwriter(s) participating in any disposition pursuant to such registration statement, and any attorney or accountant or other agent retained by any such underwriter or selected by the selling Holders, all financial and other records, pertinent corporate documents, and properties of the Company, and cause the Company's officers, directors, employees, and independent accountants to supply all information reasonably requested by any such seller, underwriter, attorney, accountant, or agent, in each case, as necessary or advisable to verify the accuracy of the information in such registration statement and to conduct appropriate due diligence in connection therewith;

(i) notify each selling Holder, promptly after the Company receives notice thereof, of the time when such registration statement has been declared effective or a supplement to any prospectus forming a part of such registration statement has been filed; and

(j) after such registration statement becomes effective, notify each selling Holder of any request by the SEC that the Company amend or supplement such registration statement or prospectus.

In addition, the Company shall ensure that, at all times after any registration statement covering a public offering of securities of the Company under the Securities Act shall have become effective, its insider trading policy shall provide that the Company's directors may implement a trading program under Rule 10b5-1 of the Exchange Act.

2.5. Furnish Information. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Registrable

Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Registrable Securities held by it, and the intended method of disposition of such securities as is reasonably required to effect the registration of such Holder's Registrable Securities.

2.6. Expenses of Registration. All expenses (other than Selling Expenses) incurred in connection with registrations, filings, or qualifications pursuant to Section 2, including all registration, filing, and qualification fees; printers' and accounting fees; fees and disbursements of counsel for the Company; and the reasonable fees and disbursements, not to exceed $50,000 (per registration), of one outside counsel for the selling Holders ("**Selling Holder Counsel**"), shall be borne and paid by the Company; provided, however, that the Company shall not be required to pay for any expenses of any registration proceeding begun

pursuant to Subsection 2.1 if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to be registered (in which case all selling Holders shall bear such expenses pro rata based upon the number of Registrable Securities that were to be included in the withdrawn registration), unless the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Subsection 2.1(a) or Subsection 2.1(b), as the case may be; provided further that if, at the time of such withdrawal, the Holders shall have learned of a material adverse change in the condition, business, or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness after learning of such information then the Holders shall not be required to pay any of such expenses and shall not forfeit their right to one registration pursuant to Subsection 2.1(a) or Subsection 2.1(b). All Selling Expenses relating to Registrable Securities registered pursuant to this Section 2 shall be borne and paid by the Holders pro rata on the basis of the number of Registrable Securities registered on their behalf.

2.7. Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any registration pursuant to this Agreement as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

2.8. Indemnification. If any Registrable Securities are included in a registration statement under this Section 2:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each selling Holder, and the partners, members, officers, directors, and stockholders of each such Holder; legal counsel, accountants and investment advisers for each such Holder; any underwriter (as defined in the Securities Act) for each such Holder; and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the Exchange Act, against any Damages, and the Company will pay to each such Holder, underwriter, controlling Person, or other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Subsection 2.8(a) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Company, which consent shall not be unreasonably withheld, nor shall the Company be liable for any Damages to the extent that they arise out of or are based upon actions or omissions made in

reliance upon and in conformity with written information furnished by or on behalf of any such Holder, underwriter, controlling Person, or other aforementioned Person expressly for use in connection with such registration.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, and each of its directors, each of its officers who has signed the registration statement, each Person (if any), who controls the Company within the meaning of the Securities Act, legal counsel and accountants for the Company, any underwriter (as defined in the Securities Act), any other Holder selling securities in such registration statement, and any controlling Person of any such underwriter or other Holder, against any Damages, in each case only to the extent that such Damages arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of such selling Holder expressly for use in connection with such registration; and each such selling Holder will pay to the Company and each other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Subsection 2.8(b) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Holder, which consent shall not be unreasonably withheld; and provided further that in no event shall the aggregate amounts payable by any Holder by way of indemnity or contribution under Subsections 2.8(b) and 2.8(d) exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of fraud or willful misconduct by such Holder.

(c) Promptly after receipt by an indemnified party under this Subsection 2.8 of notice of the commencement of any action (including any governmental action) for which a party may be entitled to indemnification hereunder, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Subsection 2.8, give the indemnifying party notice of the commencement thereof. The indemnifying party shall have the right to participate in such action and, to the extent the indemnifying party so desires, participate jointly with any other indemnifying party to which notice has been given, and to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such action. The failure to give notice to the indemnifying party within a reasonable time of the commencement of any such action shall relieve such indemnifying party of any liability to the indemnified party under this Subsection 2.8, to the extent that such failure materially prejudices the indemnifying party's ability to defend such action.

(d) To provide for just and equitable contribution to joint liability under the Securities Act in any case in which either (i) any party otherwise entitled to indemnification hereunder makes a claim for indemnification pursuant to this Subsection 2.8 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification

may not be enforced in such case, notwithstanding the fact that this Subsection 2.8 provides for indemnification in such case, or (ii) contribution under the Securities Act may be required on the part of any party hereto for which indemnification is provided under this Subsection 2.8, then, and in each such case, such parties will contribute to the aggregate losses, claims, damages, liabilities, or expenses to which they may be subject (after contribution from others) in such proportion as is appropriate to reflect the relative fault of each of the indemnifying party and the indemnified party in connection with the statements, omissions, or other actions that resulted in such loss, claim, damage, liability, or expense, as well as to reflect any other relevant equitable considerations. The relative fault of the indemnifying party and of the indemnified party shall be determined by reference to, among other things, whether the untrue or allegedly untrue statement of a material fact, or the omission or alleged omission of a material fact, relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information, and opportunity to correct or prevent such statement or omission; provided, however, that, in any such case, (x) no Holder will be required to contribute any amount in excess of the public offering price of all such Registrable Securities offered and sold by such Holder pursuant to such registration statement, and (y) no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Subsection 2.8(d), when combined with the amounts paid or payable by such Holder pursuant to Subsection 2.8(b), exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of willful misconduct or fraud by such Holder.

(e) Notwithstanding the foregoing, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, the provisions in the underwriting

agreement shall control; provided, however, that the failure of the underwriting agreement to provide for or address a matter provided for or addressed by the foregoing provisions shall not be a conflict between the foregoing provisions and the provisions in the underwriting agreement and, in such event, the foregoing provisions shall control.

(f) Unless otherwise superseded by an underwriting agreement entered into in connection with the underwritten public offering, the obligations of the Company and Holders under this Subsection 2.8 shall survive the completion of any offering of Registrable Securities in a registration under this Section 2, and otherwise shall survive the termination of this Agreement.

2.9. Reports Under Exchange Act. With a view to making available to the Holders the benefits of SEC Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company shall:

(a) make and keep available adequate current public information, as those terms are understood and defined in SEC Rule 144, at all times after the effective date of the registration statement filed by the Company for the IPO;

(b) use commercially reasonable efforts to file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the Exchange Act (at any time after the Company has become subject to such reporting requirements); and

(c) furnish to any Holder, so long as the Holder owns any Registrable Securities, forthwith upon request (i) to the extent accurate, a written statement by the Company that it has complied with the reporting requirements of SEC Rule 144 (at any time after ninety (90) days after the effective date of the registration statement filed by the Company for the IPO), the Securities Act, and the Exchange Act (at any time after the Company has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after the Company so qualifies); and (ii) such other information as may be reasonably requested in availing any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration (at any time after the Company has become subject to the reporting requirements under the Exchange Act) or pursuant to Form S-3 (at any time after the Company so qualifies to use such form).

2.10. Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of (i) prior to the IPO, (A) Holders of a majority of the Series Four Senior Preferred Stock then held by all Holders and (B) Holders of a majority of the Series Five Junior Preferred Stock then held by all Holders, in the case of each of clauses (A) and (B), consenting as separate classes, and (ii) following the IPO, the Holders of a majority of the Registrable Securities then outstanding, enter into any agreement with any holder or prospective holder of any securities of the Company that would (i) provide to such holder the right to include securities in any registration on other than either a pro rata basis with respect to the Registrable Securities or on a subordinate basis after all Holders have had the opportunity to include in the registration and offering all shares of Registrable Securities that they wish to so include or (ii) allow such holder or prospective holder to initiate a demand for registration of any securities held by such holder or prospective holder; provided that this limitation shall not apply to any additional Investor who becomes a party to this Agreement in accordance with Subsection 6.9.

2.11. "Market Stand-off" Agreement. Each Holder hereby agrees that it will not, without the prior written consent of the managing underwriter, during the period commencing on the date of the final prospectus relating to the IPO and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days, or such other period as may be requested by the Company or an underwriter to accommodate regulatory restrictions on (1) the publication or other distribution of research reports and (2) analyst recommendations and opinions, including, but not limited to, the restrictions contained in NASD Rule 2711(f)(4) of the Financial Industry Regulatory Authority or NYSE Rule 472(f)(4), or any successor provisions or amendments thereto, in each case, to the extent then in effect), (i) lend; offer; pledge; sell; contract to sell; sell any option or contract to purchase; purchase any option or contract to sell; grant any option, right, or warrant to purchase; or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable (directly or indirectly) for Common Stock (whether such shares or any such securities are then owned by the Holder or are thereafter acquired) or (ii) enter into any swap or other arrangement that transfers to another, in whole or in

part, any of the economic consequences of ownership of such securities, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash, or otherwise. The foregoing provisions of this Subsection 2.11 shall apply only to the IPO, shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, or the transfer of any shares to any trust for the direct or indirect benefit of the Holder or the immediate family of the Holder, provided that the trustee of the trust agrees to be bound in writing by the restrictions set forth herein, and provided further that any such transfer shall not involve a disposition for value, and shall be applicable to the Holders only if all executive officers and directors of the Company are subject to the same restrictions and the Company uses commercially reasonable efforts to obtain a similar agreement from all stockholders individually owning more than five percent (5%) of the Company's outstanding Common Stock (after giving effect to conversion into Common Stock of all outstanding convertible securities). The underwriters in connection with such registration are intended third-party beneficiaries of this Subsection 2.11 and shall have the right, power, and authority to enforce the provisions hereof as though they were a party hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the underwriters in connection with such registration that are consistent with this Subsection 2.11 or that are necessary to give further effect thereto.

2.12. Restrictions on Transfer.

(a) The Preferred Stock and the Registrable Securities shall not be sold, pledged, or otherwise transferred, and the Company shall not recognize and shall issue stop-transfer instructions to its transfer agent with respect to any such sale, pledge, or transfer, except upon the conditions specified in this Agreement, which conditions are intended to ensure compliance with the provisions of the Securities Act. A transferring Holder will cause any proposed purchaser, pledgee, or transferee of the Preferred Stock and the Registrable Securities held by such Holder to agree to take and hold such securities subject to the provisions and upon the conditions specified in this Agreement.

(b) Each certificate or instrument representing (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii), upon any stock split, stock dividend, recapitalization, merger, consolidation, or similar event, shall (unless otherwise permitted by the provisions of Subsection 2.12(e)) be stamped or otherwise imprinted with a legend substantially in the following form:

THE SECURITIES REPRESENTED HEREBY HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. SUCH SHARES MAY NOT BE SOLD, PLEDGED, OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR A VALID EXEMPTION FROM THE REGISTRATION AND PROSPECTUS DELIVERY REQUIREMENTS OF SAID ACT.

THE SECURITIES REPRESENTED HEREBY MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY

AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

(c) Each notice containing information required to be set forth pursuant to Section 202(a) of the General Corporation Law of the State of Delaware with respect to (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii), upon any stock split, stock dividend, recapitalization, merger, consolidation, or similar event, shall (unless otherwise permitted by the provisions of Subsection 2.12(e)) contain a notation substantially in the following form:

THE SECURITIES DESCRIBED HEREIN HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. SUCH SHARES MAY NOT BE SOLD, PLEDGED, OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR A VALID EXEMPTION FROM THE REGISTRATION AND PROSPECTUS DELIVERY REQUIREMENTS OF SAID ACT.

THE SECURITIES DESCRIBED HEREIN MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

(d) The Holders consent to the Company making a notation in its records and giving instructions to any transfer agent of the Restricted Securities in order to implement the restrictions on transfer set forth in this Subsection 2.12.

(e) The holder of each certificate representing Restricted Securities, by acceptance thereof, and the holder in whose name each book-entry representing Restricted Securities is made in the Company's stock ledger, by accepting the issuance and/or transfer thereof, agrees to comply in all respects with the provisions of this Section 2.12. Before any proposed sale, pledge, or transfer of any Restricted Securities, unless there is in effect a registration statement under the Securities Act covering the proposed transaction, the Holder thereof shall give notice to the Company of such Holder's intention to effect such sale, pledge, or transfer. Each such notice shall describe the manner and circumstances of the proposed sale, pledge, or transfer in sufficient detail and, if reasonably requested by the Company, shall be accompanied at such Holder's expense by either (i) a written opinion of legal counsel who shall, and whose legal opinion shall, be reasonably satisfactory to the Company, addressed to the Company, to the effect that the proposed transaction may be effected without registration under the Securities Act; (ii) a "no action" letter from the SEC to the effect that the proposed sale, pledge, or transfer of such Restricted Securities without registration will not result in a recommendation by the staff of the SEC that action be taken with respect thereto; or (iii) any other evidence reasonably satisfactory to counsel to the Company to the effect that the proposed sale, pledge, or transfer of the Restricted Securities may be effected without registration under the Securities Act, whereupon the Holder of such Restricted Securities shall be entitled to sell, pledge, or transfer such Restricted Securities in accordance with the terms of the notice given by the Holder to the Company. The Company will not require such a legal opinion or "no action"

letter (x) in any transaction in compliance with SEC Rule 144 or (y) in any transaction in which such Holder distributes Restricted Securities to an Affiliate of such Holder for no consideration; provided that each transferee agrees in writing to be subject to the terms of this Subsection 2.12. Each certificate or instrument evidencing the Restricted Securities transferred as above provided shall bear, except if such transfer is made pursuant to SEC Rule 144, the appropriate restrictive legend set forth in Subsection 2.12(b), except that such certificate shall not bear such restrictive legend if, in the opinion of counsel for such Holder and the Company, such legend is not required in order to establish compliance with any provisions of the Securities Act.

2.13. Termination of Registration Rights. The right of any Holder to request registration or inclusion of Registrable Securities in any registration pursuant to Subsection 2.1 or Subsection 2.2 shall terminate upon the earliest to occur of:

(a) the closing of a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation;

(b) such time as SEC Rule 144 or another similar exemption under the Securities Act is available for the sale of all of such Holder's shares without limitation during a three-month period without registration and without the requirement that the Company have made the reports required to be filed under SEC Rule 144(c)(1); and

(c) the third anniversary of the IPO resulting in the conversion of all outstanding shares of Preferred Stock.

3. Information and Observer Rights.

3.1. Delivery of Financial Statements. The Company shall deliver to each Major Investor:

(a) as soon as available and in any event within 45 days after the end of each of the first three quarters of each fiscal year of the Company, a consolidated balance sheet of the Company and its subsidiaries as of the end of such quarter and the related statements of income and stockholders' equity and of cash flows of the Company for the period commencing at the end of the previous fiscal year and ending with the end of such quarter, setting forth in each case in comparative form the corresponding figures for the corresponding period of the preceding fiscal year and the budget for such current year, all in reasonable detail and prepared in accordance with GAAP consistently applied, and duly certified (subject to year-end audit adjustments) by the chief financial officer of the Company;

(b) as soon as available and in any event within 120 days after the end of each fiscal year of the Company, a copy of the annual audit report for such year for the Company, including therein a consolidated balance sheet of the Company and its subsidiaries as of the end of such fiscal year and statements of income and stockholders' equity and of cash flows of the Company for such fiscal year, setting forth in each case in comparative form the

corresponding figures for the preceding fiscal year, all duly certified by independent public accountants of recognized standing acceptable to the Major Investors holding a majority of the Registrable Securities then held by all Major Investors;

(c) promptly after sending or making available, and only to the extent available and requested in writing, such other reports and other financial statements as the Company shall send or make available to the management of the Company from time to time;

(d) promptly upon receipt thereof, any written report submitted to the Company by independent public accountants in connection with an annual or interim audit of the books of the Company and its subsidiaries made by such accountants;

(e) promptly after sending, making available, or filing the same, such reports and financial statements as the Company shall send or make available to the stockholders of the Company; and

(f) as soon as available in the form approved by the Board of Directors, and in any event before the beginning of the fiscal year to which it applies, the annual budget and business plan of the Company.

Neither the foregoing provisions of this Section nor any other provision of this Agreement shall be in limitation of any rights which a Major Investor may have with respect to the books and records of the Company and its subsidiaries, or to inspect their properties or discuss their affairs, finances and accounts, under the laws of the jurisdictions in which they are incorporated.

Notwithstanding anything else in this Subsection 3.1 to the contrary, the Company may cease providing the information set forth in this Subsection 3.1 during the period starting with the date sixty (60) days before the Company's good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; provided that the Company's covenants under this Subsection 3.1 shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

3.2. Inspection. The Company shall permit each Major Investor, at such Major Investor's expense, to visit and inspect the Company's properties; examine its books of account and records; and discuss the Company's affairs, finances, and accounts with its executive officers, during normal business hours of the Company as may be reasonably requested by the Major Investor; provided, however, that the Company shall not be obligated pursuant to this Subsection 3.2 to provide access to any information that it reasonably and in good faith considers to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement, in form acceptable to the Company) or the disclosure of which would adversely affect the Company's attorney-client privilege regarding communications between the Company and its counsel.

3.3. Observer Rights. As long as any Designated Investor, together with such Designated Investor's Affiliates, continues to own beneficially such Designated Investor's Minimum Threshold of Preferred Stock, the Company shall invite a representative of such Designated Investor to attend all meetings of its Board of Directors in a nonvoting observer capacity and, in this respect, shall give such representative copies of all notices, minutes,

consents, and other materials that it provides to its directors at the same time and in the same manner as provided to such directors; provided, however, that such representative shall agree to hold in confidence and trust and to act in a fiduciary manner with respect to all information so provided; and provided further, that the Company reserves the right to withhold any information and to exclude such representative from any meeting or portion thereof if access to such information or attendance at such meeting could adversely affect the attorney-client privilege between the Company and its counsel or result in disclosure of trade secrets or a conflict of interest, or if such Investor or its representative is a competitor of the Company.

3.4. Termination of Information and Observer Rights. The covenants set forth in Subsection 3.1, Subsection 3.2 and Subsection 3.3 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first.

3.5. Confidentiality. Each Investor agrees that such Investor will keep confidential and will not disclose, divulge, or use for any purpose (other than to monitor its investment in the Company) any confidential information obtained from the Company pursuant to the terms of this Agreement (including notice of the Company's intention to file a registration statement), unless such confidential information (a) is known or becomes known to the public in general (other than as a result of a breach of this Subsection 3.5 by such Investor), (b) is or has been independently developed or conceived by the Investor without use of the Company's confidential information, or (c) is or has been made known or disclosed to the Investor by a third party without a breach of any obligation of confidentiality such third party may have to the Company; provided, however, that an Investor may disclose confidential information (i) to its attorneys, accountants, consultants, investment advisers and other professionals to the extent necessary to obtain their services in connection with monitoring its investment in the Company; (ii) to any prospective purchaser of any Registrable Securities from such Investor, if such prospective purchaser agrees to be bound by the provisions of this Subsection 3.5; (iii) to any Affiliate, partner, member, stockholder, or wholly-owned subsidiary of such Investor in the ordinary course of business, provided that such Investor informs such Person that such information is confidential and directs such Person to maintain the confidentiality of such information; or (iv) as may otherwise be required by law, provided that the Investor promptly notifies the Company of such disclosure and takes reasonable steps to minimize the extent of any such required disclosure.

4. Rights to Future Stock Issuances.

4.1. Right of First Offer. Subject to the terms and conditions of this Subsection 4.1 and applicable securities laws, if the Company proposes to offer or sell any New Securities, the Company shall first offer such New Securities to each Major Investor. Each Major Investor shall be entitled to apportion its rights under this Section 4, in such proportions as it deems appropriate, among itself and its Affiliates.

(a) The Company shall give notice (the "**Offer Notice**") to each Major Investor, stating (i) its bona fide intention to offer such New Securities, (ii) the number of such

New Securities to be offered, and (iii) the price and terms, if any, upon which it proposes to offer such New Securities.

(b) By notification to the Company within twenty (20) days after the Offer Notice is given, each Major Investor may elect to purchase or otherwise acquire, at the price and on the terms specified in the Offer Notice, up to that portion of such New Securities set aside by the Board of Directors for purchase by the Major Investors (which portion shall be no less than eighty percent (80%) of such New Securities), which equals the proportion that the Preferred Stock (calculated on an as-converted to Common Stock basis) then held by such Major Investor bears to the total outstanding Preferred Stock (calculated on an as-converted to Common Stock basis) then held by all Major Investors. At the expiration of such twenty (20) day period, the Company shall promptly notify each Major Investor that elects to purchase or acquire all the shares available to it (each, a "**Fully Exercising Investor**") of any other Major Investor's failure to do likewise. During the ten (10) day period commencing after the Company has given such notice, each Fully Exercising Investor may, by giving notice to the Company, elect to purchase or acquire, in addition to the number of shares specified above, up to that portion of the New Securities for which Major Investors were entitled to subscribe but that were not subscribed for by the Major Investors which is equal to the proportion that the Preferred Stock (calculated on an as-converted to Common Stock basis) then held by such Fully Exercising Investor bears to the total Preferred Stock (calculated on an as-converted to Common Stock basis) then held by all Fully Exercising Investors who wish to purchase such unsubscribed shares. The closing of any sale pursuant to this Subsection 4.1(b) shall occur within the later of one hundred and twenty 120) days of the date that the Offer Notice is given and the date of initial sale of New Securities pursuant to Subsection 4.1(c).

(c) If all New Securities referred to in the Offer Notice are not elected to be purchased or acquired as provided in Subsection 4.1(b), the Company may, during the ninety (90) day period following the expiration of the periods provided in Subsection 4.1(b), offer and sell the remaining unsubscribed portion of such New Securities to any Person or Persons at a price not less than, and upon terms no more favorable to the offeree than, those specified in the Offer Notice. If the Company does not enter into an agreement for the sale of the New Securities within such period, or if such agreement is not consummated within thirty (30) days of the execution thereof, the right provided hereunder shall be deemed to be revived and such New Securities shall not be offered unless first reoffered to the Major Investors in accordance with this Subsection 4.1.

(d) The right of first offer in this Subsection 4.1 shall not be applicable to (i) Exempted Securities (as defined in the Company's Certificate of Incorporation); (ii) shares of Common Stock issued in the IPO; (iii) the issuance of shares of Series Four Senior Preferred Stock pursuant to the Subscription Agreement, (iv) the issuance of shares of Series Four Senior Preferred Stock pursuant to outstanding convertible promissory notes issued by the Company prior to the date of this Agreement; (v) the issuance of Former Preferred Stock, as such term is defined in the Company's Certificate of Incorporation, upon the automatic exercise of outstanding warrants for the purchase of Former Preferred Stock issued by the Company prior to the date of this Agreement; and (vi) the Recapitalization, as such term is defined in the Company's Certificate of Incorporation.

(e) The rights of the Major Investors to purchase New Securities under this Section 4.1 may be waived in accordance with Section 6.6 hereof; provided, however, that in the event that the rights of Major Investors to purchase New Securities under this Section 4.1 are waived for a particular offering or sale of New Securities (a "**Waived Offering**"), the Company shall offer to each Major Investor the opportunity to purchase such Major Investor's Ratable Share of the portion of New Securities, if any, that has been set aside by the Board of Directors for purchase by existing stockholders in such Waived Offering (the "**Reserved New Securities**")); it being understood and agreed that this Section 4.1(e) shall apply if any existing stockholder is offered the right to participate as an investor in a Waived Offering. Offers made by the Company pursuant to the proviso in the immediately preceding sentence shall be communicated by the Company by means of a written offer notice (a "**Section 4.1(e) Notice**") containing information relating to (i) the number of New Securities (including the Reserved New Securities) being offered and (ii) the price and terms upon which the New Securities (including the Reserved New Securities) are being offered, and which Section 4.1(e) Notice shall indicate that it is being delivered in connection with a Waived Offering; provided, however, that a Major Investor exercising its right to purchase its Ratable Share of Reserved New Securities must do so by delivering written notice to the Company no later than 5:00 p.m., New York City time on the third (3rd) business day following receipt by such Major Investor of the applicable Section 4.1(e) Notice. The Company may allocate any unsubscribed portion of Reserved New Securities in accordance with such procedures as it may agree to with the Major Investors that have elected to purchase or acquire their full Ratable Share of the Reserved New Securities.

For purposes of this Section 4.1(e), a Major Investor's "**Ratable Share**" shall be a fraction equal to the proportion that the Preferred Stock (calculated on an as-converted to Common Stock basis) then held by such Major Investor bears to the total outstanding Preferred Stock (calculated on an as-converted to Common Stock basis) then held by all Major Investors.

(f) The rights of first offer and other participation rights set forth in this Subsection 4.1 shall terminate with respect to any Major Investor who fails to purchase, in any transaction subject to this Subsection 4.1, all of such Major Investor's pro rata amount of the New Securities allocated (or, if less than such Major Investor's pro rata amount is offered by the Company, such lesser amount so offered) to such Major Investor pursuant to this Subsection 4.1. Following any such termination, such Investor shall no longer be deemed a "Major Investor" for any purpose of this Subsection 4.1.

4.2. Termination. The covenants set forth in Subsection 4.1 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO resulting in the conversion of all outstanding shares of Preferred Stock, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first and, as to each Major Investor, in accordance with Subsection 4.1(f).

5. Additional Covenants.

5.1. Confidentiality and Assignment of Invention Provisions. Other than as may be approved by the Board of Directors from time-to-time on a case-by-case basis, the

Company shall require all officers, employees, contractors and consultants who may have access to Company Intellectual Property (as defined in the Subscription Agreement) to execute and deliver an agreement providing for, inter alia, (i) the maintenance in confidence of all Company confidential information, (ii) the disclosure and assignment to the Company, or, in the case of consultants and contractors, the disclosure and assignment, licensing or optioning, of all inventions and other intellectual property developed by such person in the context of such person's relationship with the Company or with use of any Company Intellectual

Property or confidential information, (iii) the cooperation of such person with the Company in the protection of such inventions and intellectual property rights, and (iv) the naming of the Company or its officers as attorneys-in-fact to complete any documentation necessary for the above in the case of assignments, exclusive licenses or exclusive options; provided, however, that in the case of contractors or consultants who are universities, federal research institutes, or other entities that receive government funding, or individuals or entities working for such entities, the preceding terms may be limited to the extent necessary to comply with the applicable requirements imposed on such agreements by such entities in compliance with applicable law and regulation.

5.2. Use of Name. Except where required by law or regulation, the Company shall not use the name of any Investor or any investment adviser of any Investor in any press release or other public disclosure without the advance written consent of the relevant Investor or investment adviser. The foregoing notwithstanding, the previous sentence of this Subsection 5.2 shall not be interpreted to require the Company to obtain the advance consent of any Investor in order to identify to potential third party investors in a private offering in compliance with the Securities Act the fact that such Investor is an investor in the Company.

5.3. Termination of Covenants. The covenants set forth in this Section 5 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first.

6. Miscellaneous.

6.1. Successors and Assigns. The rights under this Agreement may be assigned (but only with all related obligations) by a Holder to a transferee of Registrable Securities that (i) is an Affiliate of a Holder; (ii) is a Holder's Immediate Family Member or trust for the benefit of an individual Holder or one or more of such Holder's Immediate Family Members; or (iii) after such transfer, holds at least a majority of such Registrable Securities (subject to appropriate adjustment for stock splits, stock dividends, combinations, and other recapitalizations); provided, however, that (x) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee and the Registrable Securities with respect to which such rights are being transferred; and (y) such transferee agrees in a written instrument delivered to the Company to be bound by and subject to the terms and conditions of this Agreement, including the provisions of Subsection 2.11. For the purposes of determining the number of shares of Registrable Securities held by a transferee, the holdings of a transferee (1) that is an Affiliate or stockholder of a Holder; (2) who is a Holder's Immediate Family Member; or (3) that is a trust for the benefit of an individual Holder or such Holder's Immediate Family Member shall be aggregated together and with those of the

transferring Holder; provided further that all transferees who would not qualify individually for assignment of rights shall have a single attorney-in-fact for the purpose of exercising any rights, receiving notices, or taking any action under this Agreement. The terms and conditions of this Agreement inure to the benefit of and are binding upon the respective successors and permitted assignees of the parties. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and permitted assignees any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided herein.

6.2. Governing Law. This Agreement shall be governed by the internal laws of the State of Delaware.

6.3. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf attachment) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes**.**

6.4. Titles and Subtitles. The titles and subtitles used in this Agreement are for convenience only and are not to be considered in construing or interpreting this Agreement.

6.5. Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address set forth below or to such other address as a party may designate, and shall be either (i) delivered by hand, (ii) made by electronic mail or facsimile transmission, (iii) sent by nationally recognized overnight courier, or (iv) sent by registered or certified mail, return receipt requested, postage prepaid; provided, however, that if the notice being provided under this Section 6.5 is to an address outside of the United States, such notice shall be sent using the methods specified in (ii) and (iii) above.

If to the Company:	PTC Therapeutics, Inc. 100 Corporate Court Middlesex Business Center South Plainfield, NJ 07080-2449 Attn.: Legal Department
with a copy to (which copy shall not constitute notice):	Wilmer Cutler Pickering Hale and Dorr LLP 7 World Trade Center 250 Greenwich Street New York, NY 10007 Attn: Brian A. Johnson
and an email copy to:	legal@ptcbio.com

If to an Investor:	To the addresses set forth on each Investor's signature page attached hereto or as has been otherwise provided to the Company in writing, and as the same may be updated from time to time based on information provided by such Investor
with a copy to (which copy shall not constitute notice):	Choate, Hall & Stewart LLP Two International Place Boston, MA 02110 Attn: Brian P. Lenihan

with a copy to (which copy shall not constitute notice):	Cooley LLP 3175 Hanover Street Palo Alto, CA 94304-1130 Attn: Mehdi Khodadad

Following the IPO, for each Investor listed on Schedule B hereto (a "**New Investor**"), notices and other communications to such New Investor hereunder shall be sent solely to the person or department set forth on such New Investor's signature page attached hereto (as the same may be updated from time to time) and the Company shall not send notices or communications to any other person on behalf of such New Investor without the prior written consent of such person or a member of such department; provided that the foregoing restriction on the Company shall not apply with respect to any New Investor (i) in the event that any electronic notice or communication from the Company directed to the applicable person or department set forth on such New Investor's signature page attached hereto (as such information may be updated from time to time) fails or is returned undelivered or (ii) if, following a good faith effort by the Company to deliver any notice or communication to the applicable person or department set forth on such New Investor's signature page attached hereto (as such information may be updated from time to time), the New Investor does not acknowledge receipt of such notice or communication or otherwise remains unresponsive for a period of five (5) business days.

6.6. Amendments and Waivers. Any term of this Agreement may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance, and either retroactively or prospectively) only with the written consent of the Company and (i) prior to the IPO, (A) Holders of a majority of the Series Four Senior Preferred Stock and (B) Holders of a majority of the Series Five Junior Preferred Stock, in each case, then held by all Holders, in the case of each of clauses (A) and (B), consenting as separate classes, and (ii) following the IPO, Holders of a majority of the Registrable Securities then outstanding; provided that the Company may in its sole discretion waive compliance with Subsection 2.12(e) (and the Company's failure to object promptly in writing after notification of a proposed assignment allegedly in violation of Subsection 2.12(e) shall be deemed to be a waiver); and provided further that any provision hereof may be waived by any waiving party on such party's own behalf, without the consent of any other party. Notwithstanding the foregoing, (i) this Agreement may not be amended or terminated and the observance of any term hereof may not be waived with respect to any Investor without the written consent of such Investor, unless such amendment, termination, or waiver applies to all Investors in the same fashion (it being agreed that, subject to Section 4.1(e) hereof, a waiver of the provisions of Section 4.1 with respect to a particular transaction shall be deemed to apply to all Investors in the same fashion if such waiver

does so by its terms, notwithstanding the fact that certain Investors may nonetheless, by agreement with the Company, purchase securities in such transaction), (ii) no provision of this Agreement providing rights solely to Major Investors may be amended or terminated nor shall the observance of any term thereof be waived without the prior written consent of Major Investors holding a majority of the Registrable Securities held by all Major Investors and (iii) Section 5.2 may not be amended or waived with respect to any Investor or its investment adviser without the consent of the relevant Investor if such Investor or its investment adviser will, as a result of such amendment or waiver, be named in any press release or other public disclosure. The Company shall give prompt notice of any amendment or termination hereof or waiver hereunder to any party hereto that did not consent in writing to such amendment, termination, or waiver. Any amendment, termination, or waiver effected in accordance with this Subsection 6.6 shall be binding on all parties hereto, regardless of whether any such party has consented thereto. No waivers of or exceptions to any term, condition, or provision of this Agreement, in any one or more instances, shall be deemed to be or construed as a further or continuing waiver of any such term, condition, or provision.

6.7. Severability. In case any one or more of the provisions contained in this Agreement is for any reason held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality, or unenforceability shall not affect any other provision of this Agreement, and such invalid, illegal, or unenforceable provision shall be reformed and construed so that it will be valid, legal, and enforceable to the maximum extent permitted by law.

6.8. Aggregation of Stock. All shares of Registrable Securities held or acquired by Affiliates shall be aggregated together for the purpose of determining the availability of any rights under this Agreement and such Affiliated persons may apportion such rights as among themselves in any manner they deem appropriate.

6.9. Additional Investors. Notwithstanding anything to the contrary contained herein, if the Company issues additional shares of Preferred Stock after the date hereof, whether pursuant to the Subscription Agreement or otherwise, any purchaser of such shares of Preferred Stock may become a party to this Agreement by executing and delivering an additional counterpart signature page to this Agreement, and thereafter shall be deemed an "Investor" for all purposes hereunder. No action or consent by the Investors shall be required for such joinder to this Agreement by such additional Investor, so long as such additional Investor has agreed in writing to be bound by all of the obligations as an "Investor" hereunder.

6.10. Entire Agreement. This Agreement (including any Schedules and Exhibits hereto) constitutes the full and entire understanding and agreement among the parties with respect to the subject matter hereof, and any other written or oral agreement relating to the subject matter hereof existing between the parties is expressly canceled. The Company hereby represents and warrants that it has obtained sufficient consents and signatures from its stockholders to amend and restate the Prior Agreement as set forth herein. Upon the effectiveness of this Agreement, the Prior IRA shall be deemed amended and restated and superseded and replaced in its entirety by this Agreement, and shall be of no further force or effect.

6.11. Dispute Resolution. The parties (a) hereby irrevocably and unconditionally submit to the jurisdiction of the state courts of the State of Delaware and to the jurisdiction of the United States District Court for the District of Delaware for the purpose of any suit, action or other proceeding arising out of or based upon this Agreement, (b) agree not to commence any suit, action or other proceeding arising out of or based upon this Agreement except in the state courts of the State of Delaware or the United States District Court for the District of Delaware, and (c) hereby waive, and agree not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court.

WAIVER OF JURY TRIAL: EACH PARTY HEREBY WAIVES ITS RIGHTS TO A JURY TRIAL OF ANY CLAIM OR CAUSE OF ACTION BASED UPON OR ARISING OUT OF THIS AGREEMENT, THE OTHER TRANSACTION DOCUMENTS, THE SECURITIES OR THE SUBJECT MATTER HEREOF OR THEREOF. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS AGREEMENT AND THE TRANSACTIONS ASSOCIATED HEREWITH, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS (INCLUDING NEGLIGENCE), BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THIS SECTION HAS BEEN FULLY DISCUSSED BY EACH OF THE

PARTIES HERETO AND THESE PROVISIONS WILL NOT BE SUBJECT TO ANY EXCEPTIONS. EACH PARTY HERETO HEREBY FURTHER WARRANTS AND REPRESENTS THAT SUCH PARTY HAS REVIEWED THIS WAIVER WITH ITS LEGAL COUNSEL, AND THAT SUCH PARTY KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL

Each party will bear its own costs in respect of any disputes arising under this Agreement. Each of the parties to this Agreement consents to personal jurisdiction for any equitable action sought in the U.S. District Court for the District of Delaware or any court of the State of Delaware having subject matter jurisdiction.

6.12. Delays or Omissions. No delay or omission to exercise any right, power, or remedy accruing to any party under this Agreement, upon any breach or default of any other party under this Agreement, shall impair any such right, power, or remedy of such nonbreaching or nondefaulting party, nor shall it be construed to be a waiver of or acquiescence to any such breach or default, or to any similar breach or default thereafter occurring, nor shall any waiver of any single breach or default be deemed a waiver of any other breach or default theretofore or thereafter occurring. All remedies, whether under this Agreement or by law or otherwise afforded to any party, shall be cumulative and not alternative.

6.13. Acknowledgment. The Company acknowledges that the Investors and/or such Investors' investment advisers are in the business of making, selecting and/or managing venture capital and other investments and therefore review the business plans and related proprietary information of many enterprises, including enterprises which may have products or

services which compete directly or indirectly with those of the Company. Nothing in this Agreement shall preclude or in any way restrict the Investors and/or such Investors' investment advisers from investing or participating in any particular enterprise whether or not such enterprise has products or services which compete with those of the Company.

[Remainder of Page Intentionally Left Blank]

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

PTC THERAPEUTICS, INC.

By: /s/ Stuart W. Peltz
Name: Stuart W. Peltz
Title: Chief Executive Officer

[Investors executed by means of counterpart signature pages, the form of which is attached hereto]

[Signature Page to Second Amended and Restated Investors' Rights Agreement]

INVESTOR:

By: _____
Name: _____
Title: _____

[Signature Page to Second Amended and Restated Investors' Rights Agreement]

SCHEDULE A

Investors

Credit Suisse First Boston Equity Partners, L.P. and affiliates
Section Six Partners, L.P.
HBM Healthcare Investments (Cayman) Ltd.
Vulcan Ventures Inc. and affiliates
Delphi Ventures and affiliates
Celgene European Investment Company and affiliates
The Bay City Capital Fund III, L.P. and affiliates
Birchmere Ventures III LP
Novo A/S
Healthcap 1999 KB and affiliates

Pfizer International LLC
The Column Group, LP
Genavent Partners LP
Gilead Palo Alto, Inc. (f/k/a/ CV Therapeutics, Inc.)
Amgen SF, LLC and affiliates
Manufacturers Life Insurance
General Electric Capital Corporation
Deutsche Bank Nominees (Jersey) Limited a/c HAML
Pictet Funds (LUX)
Coller International Partners IV Limited
CDIB Biotech USA Investment, Co., Ltd.
POSCO BioVentures I, L.P.
Agron Ltd. Inc.
Tenue Business S.A.
Plaza St Petersburg Holdings Ltd
Marbre Services Limited
Frederick Frank
Security Pacific Finance, Ltd.
Minglewood Management Ltd.
Edward Spencer-Churchill
Wolvercote Investments Limited (BVI)
Leman Management Nominees
The Swanson Family Fund, Ltd.
Stronghold Capital Ltd (formerly Three Crowns Capital (Cayman) Ltd.)
Mintz Levin Investments LLC
The Board of Trustees of the Leland Stanford Junior University (DAPER I)
HSBC Trustee (C.I.) Limited
Great China Trading Company
Tichenor Ventures, LLC
Novartis BioVentures Ltd.
Oxford Finance Funding I, LLC
Oxford Finance, LLC
Midcap Financial LLC

SCHEDULE B

New Investors

Brookside Mezzanine Capital Partners Fund, L.P.
Prudential Health Sciences Fund #296227
Jennison Global Healthcare Master Fund # 002414605
Longwood Fund LP
GHC Umbrella Trust - Global Health Care Opportunity Unit Trust
North River Partners, L.P.
North River Investors (Bermuda) L.P.
Salthill Partners, L.P.
Salthill Investors (Bermuda) L.P.
Hawkes Bay Master Investors (Cayman) LP
Adage Capital Partners, LP
Ayer Capital Partners Master Fund, L.P.
Ayer Capital Partners Kestrel Fund, LP
Epworth — Ayer Capital
Ayer Special Situations Fund I, LP

Exhibit 10.1

PTC THERAPEUTICS, INC.
EIGHTH AMENDED AND RESTATED
1998 EMPLOYEE, DIRECTOR AND CONSULTANT STOCK OPTION PLAN

1. DEFINITIONS. Unless otherwise specified or unless the context otherwise requires, the following terms, as used in this Eighth Amended and Restated PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan, have the following meanings:

Administrator means the Board of Directors, unless it has delegated power to act on its behalf to the Committee, in which case the Administrator means the Committee.

Affiliate means a corporation which, for purposes of Section 424 of the Code, is a parent or subsidiary of the Company, direct or indirect.

Board of Directors means the Board of Directors of the Company.

Certificate means an Option Certificate.

Code means the United States Internal Revenue Code of 1986, as amended.

Committee means the committee of the Board of Directors to which the Board of Directors has delegated power to act under or pursuant to the provisions of the Plan.

Common Stock means shares of the Company's common stock, $.001 par value per share.

Company means PTC Therapeutics, Inc., a Delaware corporation.

Disability or Disabled means permanent and total disability as defined in Section 22(e)(3) of the Code.

Fair Market Value of a Share of Common Stock means:

(a) If the Common Stock is listed on a national securities exchange or traded in the over-the-counter market and sales prices are regularly reported for the Common Stock, the closing or last price of the Common Stock on the Composite Tape or other comparable reporting system for the trading day immediately preceding the applicable date;

(b) If the Common Stock is not traded on a national securities exchange but is traded on the over-the-counter market, if sales prices are not regularly reported for the Common Stock for the trading day referred to in clause (a), and if bid and asked prices for the Common Stock are regularly reported, the mean between the bid and the asked price for the Common Stock at the close of trading in the over-the-counter market for the trading day on which Common Stock was traded immediately preceding the applicable date; and

(c) If the Common Stock is neither listed on a national securities exchange nor traded in the over-the-counter market, such value as the Administrator, in good faith, shall determine.

ISO means an option meant to qualify as an incentive stock option under Section 422 of the Code.

Key Employee means any employee of the Company or of an Affiliate (including, without limitation, an employee who is also serving as an officer or director of the Company or of an Affiliate), or otherwise designated by the Administrator to be eligible to be granted one or more Options under the Plan.

Non-Qualified Option means an option which is not intended to qualify as an ISO.

Option means an ISO or Non-Qualified Option granted under the Plan.

Option Certificate means a certificate delivered to the Participant by the Company pursuant to the Plan, in such form as the Administrator shall approve, which sets forth the terms and conditions of a Stock Option Grant.

Participant means a Key Employee, director or consultant to whom one or more Options are granted under the Plan. As used herein, "Participant" shall include "Participant's Survivors" where the context requires.

Plan means this Eighth Amended and Restated PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan.

Shares means shares of the Common Stock as to which Options have been or may be granted under the Plan or any shares of capital stock into which the Shares are changed or for which they are exchanged within the provisions of Paragraph 3 of the Plan. The Shares issued upon exercise of Options granted under the Plan may be authorized and unissued shares or shares held by the Company in its treasury, or both.

Stock Option Grant means a grant of an option to purchase Shares under the Plan in either the form of an ISO or Non-Qualified Option.

Survivors means a deceased Participant's legal representatives and/or any person or persons who acquired the Participant's rights to an Option by will or by the laws of descent and distribution.

2. PURPOSES OF THE PLAN. The Plan is intended to encourage ownership of Shares by Key Employees and directors of and certain consultants to the Company in order to attract such people, to induce them to work for the benefit of the Company or of an Affiliate and to provide additional incentive for them to promote the success of the Company or of an Affiliate. The Plan provides for the granting of Stock Option Grants.

3. SHARES SUBJECT TO THE PLAN.

a. The number of Shares which may be issued from time to time pursuant to this Plan shall be equal to Three Million Nine Hundred Seventy-six Thousand Eight (3,976,008), or the equivalent of such numbers of Shares after the Administrator, in its sole discretion, has interpreted the effect of

any stock split, stock dividend, combination, recapitalization or similar transaction in accordance with Paragraph 16 of the Plan;

b. The maximum number of Shares that may be issued as ISOs pursuant to this Plan shall be equal to Three Million Nine Hundred Seventy-six Thousand Eight (3,976,008), or the equivalent of such numbers of Shares after the Administrator, in its sole discretion, has interpreted the effect of any stock split, stock dividend, combination, recapitalization or similar transaction in accordance with Paragraph 16 of the Plan; and

c. If an Option ceases to be "outstanding", in whole or in part, the Shares which were subject to such Option shall be available for the granting of other Options under the Plan. Any Option shall be treated as "outstanding" until such Option is exercised in full, or terminates or expires under the provisions of the Plan, or by agreement of the parties to the pertinent Option Certificate.

4. ADMINISTRATION OF THE PLAN. The Administrator of the Plan will be the Board of Directors, except to the extent the Board of Directors delegates its authority to the Committee, in which case the Committee shall be the Administrator. Subject to the provisions of the Plan, the Administrator is authorized to:

a. Interpret the provisions of the Plan or of any Stock Option Grant or Option Certificate and to make all rules and determinations which it deems necessary or advisable for the administration of the Plan;

b. Determine which employees of the Company or of an Affiliate shall be designated as Key Employees and which of the Key Employees, directors and consultants shall be granted Options;

c. Determine the number of Shares for which an Option or Options shall be granted, provided, however, that in no event shall Options to purchase more than 700,000 Shares be granted to any Participant in any fiscal year; and

d. Specify the terms and conditions upon which an Option or Options may be granted

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provided, however, that all such interpretations, rules, determinations, terms and conditions shall be made and prescribed in the context of preserving the tax status under Section 422 of the Code of those Options which are designated as ISOs. Subject to the foregoing, the interpretation and construction by the Administrator of any provisions of the Plan or of any Option granted under it shall be final, unless otherwise determined by the Board of Directors, if the Administrator is the Committee.

5. ELIGIBILITY FOR PARTICIPATION. The Administrator will, in its sole discretion, name the Participants in the Plan, provided, however, that each Participant must be a Key Employee, director or consultant of the Company or of an Affiliate at the time an Option is granted. Notwithstanding the foregoing, the Administrator may authorize the grant of an Option to a person not then an employee, director or consultant of the Company or of an Affiliate; provided, however, that the actual grant of such Option shall be conditioned upon such person becoming eligible to become a Participant at or prior to the time of the delivery of the Option Certificate evidencing such Option. ISOs may be granted only to Key Employees. Non-Qualified Options may be granted to any Key Employee, director or consultant of the Company or an Affiliate. The granting of any Option to any individual shall neither entitle that individual to, nor disqualify him or her from, participation in any other grant of Options.

6. TERMS AND CONDITIONS OF OPTIONS. Each Stock Option Grant shall be set forth in writing in an Option Certificate, duly executed by the Company and, to the extent required by law or requested by the Company, by the Participant. The Administrator may provide that Options be granted subject to such terms and conditions, consistent with the terms and conditions specifically required under this Plan, as the Administrator may deem appropriate including, without limitation, subsequent approval by the shareholders of the Company of this Plan or any amendments thereto.

A. Non-Qualified Options: Each Option intended to be a Non-Qualified Option shall be subject to the terms and conditions which the Administrator determines to be appropriate and in the best interest of the Company, subject to the following minimum standards for any such Non-Qualified Option:

a. Option Price: Each Option Certificate shall state the option price (per share) of the Shares covered by each Stock Option Grant, which option price shall be determined by the Administrator but shall not be less than 85% of the Fair Market Value per share of Common Stock;

b. Each Option Certificate shall state the number of Shares to which it pertains;

c. Each Option Certificate shall state the date or dates on which it first is exercisable and the date after which it may no longer be exercised, and may provide that the Option rights accrue or become exercisable in installments over a period of months or years, or upon the occurrence of certain conditions or the attainment of stated goals or events, or through other circumstances or programs approved by the Administrator (the "Vesting Provisions");

d. The provisions of Paragraph 6(A)(c) above notwithstanding, with the consent of the Administrator, the vesting provisions specified in a Participant's employment agreement shall be the Vesting Provisions that apply to the relevant Non-Qualified Options; and

e. Exercise of any Option may be conditioned upon the Participant's execution of a Share purchase agreement in form satisfactory to the Administrator providing for certain protections for the Company and its other shareholders, including requirements that:

i. The Participant's or the Participant's Survivors' right to sell or transfer the Shares may be restricted; and

ii. The Participant or the Participant's Survivors may be required to execute letters of investment intent and must also acknowledge that the Shares will bear legends noting any applicable restrictions.

B. ISOs: Each Option intended to be an ISO, in accordance with Section 422 of the Code, shall be issued only to a Key Employee and be subject to the following terms and conditions, with such additional restrictions or changes as the Administrator determines are appropriate but not in conflict with Section 422 of the Code and relevant regulations and rulings of the Internal Revenue Service:

a. Minimum standards: The ISO shall meet the minimum standards required of Non-Qualified Options, as described in Paragraph 6(A) above, except clause (a) thereunder;

b. Option Price: Immediately before the ISO is granted, if the Participant owns, directly or by reason of the applicable attribution rules in Section 424(d) of the Code:

i. Ten percent (10%) or less of the total combined voting power of all classes of stock of the Company or an Affiliate, the Option price per share of the Shares covered by each ISO shall not be less than one hundred percent (100%) of the Fair Market Value per share of the Shares on the date of the Stock Option Grant; or

ii. More than ten percent (10%) of the total combined voting power of all classes of stock of the Company or an Affiliate, the Option price per share of the Shares covered by each ISO shall not be less than one hundred ten percent (110%) of the said Fair Market Value on the date of the Stock Option Grant;

c. Term of Option: For Participants who own:

i. Ten percent (10%) or less of the total combined voting power of all classes of stock of the Company or an Affiliate, each ISO shall terminate not more than ten (10) years from the date of the Stock Option Grant or at such earlier time as the Option Certificate may provide; or

ii. More than ten percent (10%) of the total combined voting power of all classes of stock of the Company or an Affiliate, each ISO shall terminate not more than five (5) years from the date of the Stock Option Grant or at such earlier time as the Option Certificate may provide;

d. Limitation on Yearly Exercise: The Option Certificates shall restrict the amount of ISOs which may be exercisable in any calendar year (under this or any other ISO plan of the Company or an Affiliate) so that the aggregate Fair Market Value (determined at the time each ISO is granted) of the stock with respect to which ISOs are exercisable for the first time by the Participant in any calendar year does not exceed one hundred thousand dollars ($100,000), provided that this subparagraph (d) shall have no force or effect if its inclusion in the Plan is not necessary for Options issued as ISOs to qualify as ISOs pursuant to Section 422(d) of the Code; and

e. Intention to be Treated as an ISO: It is the Company's intent that an ISO qualify for the favorable tax treatment provided to holders of Options that meet the standards of Section 422 of the Code. Any provision of this Plan, an Option Certificate or any other relevant document which conflicts with the Code so that an Option intended to be an ISO would not be deemed an ISO is null and void and any ambiguities shall be resolved so that the Option qualifies as an ISO. Nonetheless, if the Option is determined not to be an ISO, the Participant shall be deemed to acknowledge and agree that neither the Company nor

any Affiliate is responsible to compensate him or her or otherwise make up for the treatment of the Option as a Non-Qualified Option and not as an ISO. The Participant is deemed to have been advised to consult with his or her own tax advisors regarding the tax effects of the Option and the requirements necessary to obtain favorable tax treatment under Section 422 of the Code, including, but not limited to, holding period requirements.

7. EXERCISE OF OPTIONS AND ISSUE OF SHARES. An Option (or any part or installment thereof) shall be exercised by giving written notice to the Company at its principal executive office address, together with provision for payment of the full purchase price in accordance with this Paragraph for the Shares as to which the Option is being exercised, and upon compliance with any other condition(s) set forth in the Option Certificate. Such written notice shall be signed by the person exercising the Option, shall state the number of Shares with respect to which the Option is being exercised and shall contain any representation required by the Plan or the Option Certificate. Payment of the purchase price for the Shares as to which such Option is being exercised shall be made (a) in United States dollars in cash or by check, or (b) at the discretion of the Administrator, through delivery of shares of Common Stock having a Fair Market Value equal as of the date of the exercise to the cash exercise price of the Option, or (c) at the discretion of the Administrator, by having the Company retain from the shares otherwise issuable upon exercise of the Option, a number of shares having a Fair Market Value equal as of the date of exercise to the exercise price of the Option, or (d) at the discretion of the Administrator, by delivery of the grantee's personal recourse note bearing interest payable not less than annually at no less than 100% of the applicable Federal rate, as defined in Section 1274(d) of the Code, or (e) at the discretion of the Administrator, in accordance with a cashless exercise program established with a securities brokerage firm, and as approved by the Administrator, or (f) at the discretion of the Administrator, by any combination of (a), (b), (c), (d) and (e) above. Notwithstanding the foregoing, the Administrator shall accept only such payment on exercise of an ISO as is permitted by Section 422 of the Code.

The Company shall then reasonably promptly deliver the Shares as to which such Option was exercised to the Participant (or to the Participant's Survivors, as the case may be). In determining what constitutes "reasonably promptly," it is expressly understood that the issuance and delivery of the Shares may be delayed by the Company in order to comply with any law or regulation (including, without limitation, state securities or "blue sky" laws) which requires the Company to take any action with respect to the Shares prior to their issuance. The Shares shall, upon delivery, be evidenced by an appropriate certificate or certificates for fully paid, non-assessable Shares.

The Administrator shall have the right to accelerate the date of exercise of any installment of any Option; provided that the Administrator shall not accelerate the exercise date of any installment of any Option granted to any Key Employee as an ISO (and not previously converted into a Non-Qualified Option pursuant to Paragraph 19) if such acceleration would violate the annual vesting limitation contained in Section 422(d) of the Code, as described in Paragraph 6.B.d.

The Administrator may, in its discretion, amend any term or condition of an outstanding Stock Option Grant provided (i) such term or condition as amended is permitted by the Plan, (ii) any such amendment shall be made only with the consent of the Participant to whom the Option was granted, or in the event of the death of the Participant, the Participant's Survivors, if the amendment is adverse to the Participant**,** and (iii) any such amendment of any ISO shall be made only after the Administrator, after consulting the counsel for the Company, determines whether such amendment would constitute a "modification" of any Option which is an ISO (as that term is defined in Section 424(h) of the Code) or would cause any adverse tax consequences for the holder of such ISO.

8. RIGHTS AS A SHAREHOLDER. No Participant to whom an Option has been granted shall have rights as a shareholder with respect to any Shares covered by such Option, except after due exercise of the Option and tender of the full purchase price, if any, for the Shares being purchased pursuant to such exercise and registration of the Shares in the Company's share register in the name of the Participant.

9. ASSIGNABILITY AND TRANSFERABILITY OF OPTIONS. By its terms, an Option granted to a Participant shall not be transferable by the Participant other than (i) by will or by the laws of descent and distribution, (ii) as approved by the Administrator in its sole discretion and set forth in the applicable Option Certificate, (iii) if approved by the Administrator in its sole discretion, through establishment of blind trusts, family limited partnerships, or other estate planning vehicles wherein the Participant or his direct descendants are the primary beneficiary, (iv) if approved by the Administrator in its sole discretion, in accordance with the division of property rights set forth in an authorized settlement agreement arising from the Participant's divorce, or (v) under any other circumstances that are approved by the Administrator in its sole discretion. Notwithstanding the foregoing, an ISO transferred in accordance with subsections 9(ii)-(v) above shall no longer qualify as an incentive stock option under Section 422 of the Code. The designation of a beneficiary of an Option by a Participant, with the prior approval of the Administrator and in such form as the Administrator shall prescribe, shall not be deemed a transfer prohibited by this Paragraph. Except as provided above, during the Participant's lifetime, an Option shall only be exercisable by such Participant (or by his or her legal representative) and shall not be assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and shall not be subject to execution, attachment or similar process. Any attempted transfer, assignment, pledge, hypothecation or other disposition of any Option or of any rights granted thereunder contrary to the provisions of this Plan, or the levy of any attachment or similar process upon an Option, shall be null and void.

The Participant is required to notify the Company in writing immediately after the Participant makes a Disqualifying Disposition of any of the Shares acquired pursuant to the exercise of the Option. A Disqualifying Disposition is defined in Section 424(c) of the Code and includes any disposition (including any sale) of such Shares before the later of (a) two years after the date the Participant was granted the Option or (b) one year after the date the Participant acquired Shares by exercising the Option, except as otherwise provided in Section 424(c) of the Code. If the Participant has died before the Shares are sold, these holding period requirements do not apply and no Disqualifying Disposition can occur thereafter.

10. EFFECT OF TERMINATION OF SERVICE OTHER THAN "FOR CAUSE" OR DEATH OR DISABILITY. Except as otherwise provided in the pertinent Option Certificate, in the event of a termination of service (whether as an employee, director or consultant) with the Company or an Affiliate before the Participant has exercised an Option, the following rules apply:

a. A Participant who ceases to be an employee, director or consultant of the Company or of an Affiliate (for any reason other than termination "for cause", Disability, or death for which events there are special rules in Paragraphs 11, 12, and 13, respectively), may exercise any Option granted to him or her (i) within three (3) months of such termination to the extent that the Option is exercisable on the date of such termination of service, but only if the Administrator has so designated in the pertinent Option Certificate, or (ii) over such other term as the Administrator shall determine in its sole discretion;

b. Except as provided in Subparagraph (c) below, or Paragraph 12 or 13, in no event may an Option Certificate provide, if an Option is intended to be an ISO, that the time for exercise be later than three (3) months after the Participant's termination of employment;

c. The provisions of this Paragraph, and not the provisions of Paragraph 12 or 13, shall apply to a Participant who subsequently becomes Disabled or dies after the termination of employment, director status or consultancy in all cases with the Company or an Affiliate, provided, however, in the case of a Participant's Disability or death within three (3) months after the termination of employment, director status or consultancy, the Participant or the Participant's Survivors may exercise the Option within one (1) year after the date of the Participant's termination of employment, but in no event after the date of expiration of the term of the Option;

d. Notwithstanding anything herein to the contrary, if subsequent to a Participant's termination of employment, termination of director status or termination of consultancy, but prior to the exercise of an Option, the Board of Directors determines that, either prior or subsequent to the Participant's termination, the Participant engaged in conduct which would constitute "cause", then such Participant shall forthwith cease to have any right to exercise any Option;

e. A Participant to whom an Option has been granted under the Plan who is absent from work with the Company or with an Affiliate because of temporary disability (any disability other than a permanent and total Disability as defined in Paragraph 1 hereof), or who is on leave of absence for any purpose, shall not, during the period of any such absence, be deemed, by virtue of such absence alone, to have terminated such Participant's employment, director status or consultancy with the Company or with an Affiliate, except as the Administrator may otherwise expressly provide; and

f. Except as required by law or as set forth in the pertinent Option Certificate, Options granted under the Plan shall not be affected by any change of a Participant's status within or among the Company and any Affiliates, so long as the Participant continues to be an employee, director or consultant of the Company or any Affiliate.

11. EFFECT OF TERMINATION OF SERVICE "FOR CAUSE". Except as otherwise provided in the pertinent Option Certificate, the following rules apply if the Participant's service (whether as an employee, director or consultant) with the Company or an Affiliate is terminated "for cause" prior to the time that all his or her outstanding Options have been exercised:

a. All outstanding and unexercised Options as of the time the Participant is notified his or her service is terminated "for cause" will immediately be forfeited;

b. In addition to any definition of the term "for cause" set forth in any employment agreement between the Company and the Participant, for purposes of this Plan, the term "cause" shall include, without limitation (i) the failure of the Participant to perform any of his material duties to the Company or any of its Affiliates, (ii) the conviction of the Participant of any felony involving moral turpitude, (iii) any acts of fraud or embezzlement by the Participant involving the Company or any of its Affiliates, (iv) violation of any federal, state or local law, or administrative regulation related to the business of the Company or any of its Affiliates, (v) a conflict of interest, (vi) conduct that could result in publicity reflecting unfavorably on the Company or any of its Affiliates in a material way, (vii) failure to comply with the policies of the Company or any of its Affiliates, (viii) the unauthorized disclosure of confidential information, or (ix) a breach of the terms of any employment agreement, confidentiality agreement, non-competition and non-solicitation agreement or any other agreement between the Participant and the Company or any of its Affiliates, after giving effect to the notification provisions, if any, and the mechanisms to remedy or cure a breach, if appropriate, as described in any such agreement. The determination of the Administrator as to the existence of "cause" will be conclusive on the Participant and the Company; and

c. "Cause" is not limited to events which have occurred prior to a Participant's termination of service, nor is it necessary that the Administrator's finding of "cause" occur prior to termination. If the Administrator determines, subsequent to a Participant's termination of service but prior to the exercise of

an Option, that either prior or subsequent to the Participant's termination the Participant engaged in conduct which would constitute "cause," then the right to exercise any Option is forfeited.

12. EFFECT OF TERMINATION OF SERVICE FOR DISABILITY. Except as otherwise provided in the pertinent Option Certificate, a Participant who ceases to be an employee, director or consultant of the Company or of an Affiliate by reason of Disability may exercise any Option granted to such Participant:

    a. To the extent that the Option has become exercisable according to the vesting period of such Option as of the date of Disability; and

    b. To the extent of a pro rata portion through the date of Disability of any additional Options that would have become exercisable on the next vesting date had the Participant not become Disabled. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of Disability.

    A Disabled Participant may exercise such rights only within the period ending one (1) year after the date of the Participant's termination of employment, directorship or consultancy, as the case may be, notwithstanding that the Participant might have been able to exercise the Option as to some or all of the Shares on a later date if the Participant had not become disabled and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

    The Administrator shall make the determination both of whether Disability has occurred and the date of its occurrence (unless a procedure for such determination is set forth in another agreement between the Company and such Participant, in which case such procedure shall be used for such determination). If requested, the Participant shall be examined by a physician selected or approved by the Administrator, the cost of which examination shall be paid for by the Company.

13. EFFECT OF DEATH WHILE AN EMPLOYEE, DIRECTOR OR CONSULTANT. Except as otherwise provided in the pertinent Option Certificate, in the event of the death of a Participant while the Participant is an employee, director or consultant of the Company or of an Affiliate, the Participant's Survivors may exercise any outstanding Option granted to the Participant:

    a. To the extent that the Option has become exercisable according to the vesting period of such Option as of the date of death; and

    b. To the extent of a pro rata portion through the date of death of any additional Options that would have become exercisable on the next vesting date had the Participant not died. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of death.

    If the Participant's Survivors wish to exercise the Option, they must take all necessary steps to exercise the Option within one (1) year after the date of death of such Participant, notwithstanding that the decedent might have been able to exercise the Option as to some or all of the Shares on a later date if he or she had not died and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

14. PURCHASE FOR INVESTMENT. Unless the offering and sale of the Shares to be issued upon the particular exercise of an Option shall have been effectively registered under the Securities Act of 1933, as now in force or hereafter amended (the "1933 Act"), the Company shall be under no obligation to issue the Shares covered by such exercise unless and until the following conditions have been fulfilled:

    a. The person(s) who exercise(s) such Option shall warrant to the Company, prior to the receipt of such Shares, that such person(s) are acquiring such Shares for their own respective accounts, for investment, and not with a view to, or for sale in connection with, the distribution of any such Shares, in which event the person(s) acquiring such Shares shall be bound by the provisions of the following legend which shall be endorsed upon the certificate(s) evidencing their Shares issued pursuant to such exercise or such grant:

    "The shares represented by this certificate have been taken for investment, and they may not be sold or otherwise transferred by any person, including a pledgee, unless (1) either (a) a Registration Statement with respect to such shares shall be effective under the Securities Act of 1933, as amended, or (b) the Company shall have received an opinion of counsel satisfactory to it that an exemption from registration under such Act is then available, and (2) there shall have been compliance with all applicable state securities laws."; and

    b. At the discretion of the Administrator, the Company shall have received an opinion of its counsel that the Shares may be issued upon such particular exercise in compliance with the 1933 Act without registration thereunder.

15. DISSOLUTION OR LIQUIDATION OF THE COMPANY. Upon the dissolution or liquidation of the Company, all Options granted under this Plan which as of such date shall not have been exercised will terminate and become null and void; provided, however, that if the rights of a Participant or a Participant's Survivors have not otherwise terminated and expired, the Participant or the Participant's Survivors will have the right immediately prior to such dissolution or liquidation to exercise any Option to the extent that the Option is exercisable as of the date immediately prior to such dissolution or liquidation.

16. ADJUSTMENTS. Upon the occurrence of any of the following events, a Participant's rights with respect to any Option granted to him or her hereunder which has not previously been exercised in full shall be adjusted as hereinafter provided, unless otherwise specifically provided in the pertinent Option Certificate or, subject to the consent of the Administrator, as otherwise specified in an employment or other agreement between the Company and the Participant:

    A. Stock Dividends and Stock Splits. If (i) the shares of Common Stock shall be subdivided or combined into a greater or smaller number of shares or if the Company shall issue any shares of Common Stock as a stock dividend on its outstanding Common Stock, or (ii) additional shares or new or different shares or other securities of the Company or other non-cash assets are distributed with respect to such shares of Common Stock, the number of shares of Common Stock deliverable upon the exercise of such Option may be appropriately increased or decreased proportionately, and appropriate adjustments may be made in the purchase price per share to reflect such events. The number of Shares subject to the limitation in Paragraph 4(c) shall also be proportionately adjusted upon the occurrence of such events.

    B. Consolidations or Mergers. If the Company is to be consolidated with or acquired by another entity in a merger, sale of all or substantially all of the Company's assets or otherwise (an "Acquisition"), the Administrator or the board of directors of any entity assuming the obligations of the Company hereunder (the "Successor Board"), shall, as to outstanding Options, either (i) make appropriate provision for the continuation of such Options by substituting on an equitable basis for the Shares then subject to such Options, including without limitation any provisions relating to the acceleration of vesting, either the consideration payable with respect to the outstanding shares of Common Stock in connection with the

Acquisition or securities of any successor or acquiring entity; or (ii) upon written notice to the Participants, provide that all vested Options must be exercised (either to the extent then exercisable, including Options subject to accelerated vesting provisions, or, at the discretion of the Administrator, all Options being made fully exercisable for purposes of this Subparagraph), at the end of which period the Options shall terminate; or (iii) terminate all Options in exchange for a cash payment equal to the excess of the Fair Market Value of the Shares subject to such Options (either to the extent then exercisable or, at the discretion of the Administrator, all Options being made fully exercisable, including Options subject to accelerated vesting provisions, for purposes of this Subparagraph) over the exercise price thereof.

C. Recapitalization or Reorganization. In the event of a recapitalization or reorganization of the Company (other than a transaction described in Subparagraph B above) pursuant to which securities of the Company or of another corporation are issued with respect to the outstanding shares of Common Stock, a Participant upon exercising an Option shall be entitled to receive for the purchase price, if any, paid upon such exercise the securities which would have been received if such Option had been exercised prior to such recapitalization or reorganization.

D. Modification of ISOs. Notwithstanding the foregoing, any adjustments made pursuant to Subparagraph A, B or C above with respect to ISOs shall be made only after the Administrator, after consulting with counsel for the Company, determines whether such adjustments would constitute a "modification" of such ISOs (as that term is defined in Section 424(h) of the Code) or would cause any adverse tax consequences for the holders of such ISOs. If the Administrator determines that such adjustments made with respect to ISOs would constitute a modification of such ISOs, it may refrain from making such adjustments, unless the holder of an ISO specifically requests in writing that such adjustment be made and such writing indicates that the holder has full knowledge of the consequences of such "modification" on his or her income tax treatment with respect to the ISO.

17. ISSUANCES OF SECURITIES. Except as expressly provided herein, no issuance by the Company of shares of stock of any class, or securities convertible into shares of stock of any class, shall affect, and no adjustment by reason thereof shall be made with respect to, the number or price of shares subject to Options. Except as expressly provided herein, no adjustments shall be made for dividends paid in cash or in property (including without limitation, securities) of the Company prior to any issuance of Shares pursuant to a Stock Option Grant.

18. FRACTIONAL SHARES. No fractional shares shall be issued under the Plan and the person exercising such right shall receive from the Company cash in lieu of such fractional shares equal to the Fair Market Value thereof.

19. CONVERSION OF ISOs INTO NON-QUALIFIED OPTIONS; TERMINATION OF ISOs. The Administrator, at the written request of any Participant, may in its discretion take such actions as may be necessary to convert such Participant's ISOs (or any portions thereof) that have not been exercised on the date of conversion into Non-Qualified Options at any time prior to the expiration of such ISOs, regardless of whether the Participant is an employee of the Company or an Affiliate at the time of such conversion. Such actions may include, but not be limited to,

extending the exercise period or reducing the exercise price of the appropriate installments of such Options. At the time of such conversion, the Administrator (with the consent of the Participant) may impose such conditions on the exercise of the resulting Non-Qualified Options as the Administrator in its discretion may determine, provided that such conditions shall not be inconsistent with this Plan. Nothing in the Plan shall be deemed to give any Participant the right to have such Participant's ISOs converted into Non-Qualified Options, and no such conversion shall occur until and unless the Administrator takes appropriate action. The Administrator, with the consent of the Participant, may also terminate any portion of any ISO that has not been exercised at the time of such conversion.

20. WITHHOLDING. In the event that any federal, state, or local income taxes, employment taxes, Federal Insurance Contributions Act ("F.I.C.A.") withholdings or other amounts are required by applicable law or governmental regulation to be withheld from the Participant's salary, wages or other remuneration in connection with the exercise of an Option or a Disqualifying Disposition (as defined in Paragraphs 9 and 21), the Company may withhold from the Participant's compensation, if any, or may require that the Participant advance in cash to the Company, or to any Affiliate of the Company which employs or employed the Participant, the statutory minimum amount of such withholdings unless a different withholding arrangement, including the use of shares of the Company's Common Stock or a promissory note, is authorized by the Administrator (and permitted by law). For purposes hereof, the fair market value of the shares withheld for purposes of payroll withholding shall be determined in the manner provided in Paragraph 1 above, as of the most recent practicable date prior to the date of exercise. If the fair market value of the shares withheld is less than the amount of payroll withholdings required, the Participant may be required to advance the difference in cash to the Company or the Affiliate employer. The Administrator in its discretion may condition the exercise of an Option for less than the then Fair Market Value on the Participant's payment of such additional withholding.

21. NOTICE TO COMPANY OF DISQUALIFYING DISPOSITION. Each Key Employee who receives an ISO must agree to notify the Company in writing immediately after the Key Employee makes a Disqualifying Disposition of any shares acquired pursuant to the exercise of an ISO. A Disqualifying Disposition is any disposition (including any sale) of such shares before the later of (a) two years after the date the Key Employee was granted the ISO, or (b) one year after the date the Key Employee acquired Shares by exercising the ISO. If the Key Employee has died before such stock is sold, these holding period requirements do not apply and no Disqualifying Disposition can occur thereafter.

22. TERMINATION OF THE PLAN. The Plan will terminate on August 12, 2008, the date which is ten (10) years from the earlier of the date of its adoption and the date of its approval by the shareholders of the Company. The Plan may be terminated at an earlier date by vote of the shareholders of the Company; provided, however, that any such earlier termination shall not affect any Option Certificates executed prior to the effective date of such termination.

23. AMENDMENT OF THE PLAN AND CERTIFICATES. The Plan may be amended by the shareholders of the Company. The Plan may also be amended by the Administrator, including, without limitation, to the extent necessary to qualify any or all outstanding Options granted under the Plan or Options to be granted under the Plan for favorable federal income tax treatment (including deferral of taxation upon exercise) as may be afforded incentive stock options under Section 422 of the Code, and to the extent necessary to qualify the shares issuable upon exercise of any outstanding Options granted, or Options to be granted, under the Plan for listing on any national securities exchange or quotation in any national automated quotation system of securities dealers. Any amendment approved by the Administrator which the Administrator determines is of a scope that requires shareholder approval shall be subject to obtaining such shareholder approval. Any modification or amendment of the Plan shall not, without the consent of a Participant, adversely affect his or her rights under an Option previously granted to him or her. With the consent of the Participant affected, the Administrator may amend outstanding Option Certificates in a manner which may be adverse to the Participant but which is not inconsistent with the Plan. In the discretion of the Administrator, outstanding Option Certificates may be amended by the Administrator in a manner which is not adverse to the Participant. Except as provided herein, the terms and provisions of any Option Certificate may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of such Option Certificate, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

24. EMPLOYMENT OR OTHER RELATIONSHIP. Nothing in this Plan or any Option Certificates shall be deemed to prevent the Company or an Affiliate from terminating the employment, consultancy or director status of a Participant, nor to prevent a Participant from terminating his or her own employment, consultancy or director status or to give any Participant a right to be retained in employment or other service by the Company or any Affiliate for any period of time.

25. GOVERNING LAW. This Plan shall be construed and enforced in accordance with the law of the State of Delaware.

PTC Therapeutics, Inc.

INCENTIVE STOCK OPTION CERTIFICATE

THIS INCENTIVE STOCK OPTION CERTIFICATE (this "Certificate"), dated as of the *XX* day of *XXXX*, *XXXX*, certifies that, pursuant to the PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan, as amended (the "Plan"), the Board of Directors of PTC Therapeutics, Inc. (the "Company") has granted an Option to purchase shares of Common Stock, $.001 par value per share (the "Shares"), of the Company, as follows:

Name of Optionee:	*XXXX*
Address of Optionee:	*XXXX*
	XXXX
Grant ID Number:	*#XXXX*
Number of Shares:	*XXXXX*
Option Price:	US $*XXX* per share
Date of Grant:	*XXXX*
Vesting Reference Date:	*XXXX*

The Option is subject to all the terms, conditions and limitations set forth in the Plan, which is incorporated herein by reference, and to the following additional terms specified by the Board of Directors of the Company. The Optionee acknowledges receipt of a copy of the Plan. All capitalized terms used in this Certificate and not otherwise defined herein shall have the respective meanings ascribed to them in the Plan.

The Option shall vest and be exercisable as follows:

(a) As to one-third (1/3) of the Shares, on the first anniversary of the Vesting Reference Date; and

(b) As to the remaining two-thirds (2/3) of the Shares (the "Remainder"), monthly thereafter, so that one-twenty-fourth (1/24) of the Remainder vests on each of the thirteenth through thirty-sixth monthly anniversaries of the Vesting Reference Date.

The Option shall terminate ten (10) years from the Date of Grant or such shorter period as set forth in the Plan in the event of the Optionee's termination of service, Death or Disability. For the avoidance of doubt, the Plan provides that in the event of a termination other than for Death, Disability, or "cause" (as defined in the Plan), the vested portion of the Option may be exercised within three (3) months after the date the Optionee ceases to be an employee of the Company, or within the originally prescribed term of the Option, whichever is shorter, but may not be exercised thereafter. Further, for the avoidance of doubt, the Plan provides that in the event of a termination for "cause" (as defined in the Plan), the Option will immediately be forfeited and the Optionee will lose all rights to exercise the Option (whether or not vested) for Shares.

The Option is not assignable or transferable, other than as provided in the Plan. Until the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934, the Optionee may exercise this Option by written notice to the Company, in substantially the form of Exhibit A attached hereto, and as provided in the Plan. Following such registration of the Shares pursuant to the Securities Exchange Act of 1934, the form of written notice for exercise of the Option shall be in a form to be approved by the Company's board of directors. No partial exercise of the Option may be for less than 100 full shares. In no event shall the Company be required to issue fractional shares.

The Optionee acknowledges that any income taxes or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Optionee's responsibility.

No Shares will be issued pursuant to the exercise of this Option unless and until the Optionee pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of such exercise of this Option. The Company may withhold from the Optionee's remuneration, if any, any withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Optionee on exercise of the Option. The Optionee further agrees that, if the Company does not withhold an amount from the Optionee's remuneration

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sufficient to satisfy the Company's income tax withholding obligation, the Optionee will reimburse the Company on demand, in cash, for the amount under-withheld prior to the issuance of the Shares upon such exercise.

The Shares acquired by the Optionee pursuant to the exercise of the Option granted hereby shall not be transferred by the Optionee except as permitted herein. The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

In the event of the Optionee's termination of service for "cause" as defined in Article 11 of the Plan, or in the event the Administrator determines, subsequent to the Optionee's termination of service, that either prior or subsequent to Optionee's termination the Optionee engaged in conduct which would constitute "cause," then the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Certificate (including, without limitation, Shares purchased after termination of employment, consultancy or directorship, Disability or death in accordance with the Plan). In the event the Company does not exercise its option pursuant to this paragraph, the restrictions set forth in the balance of this Certificate shall not thereby lapse, and the Optionee for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this paragraph:

(a) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this paragraph shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of service;

(b) The Company's option to repurchase the Optionee's Shares in the event of termination of service shall be valid for a period of eighteen (18) months commencing with the date of such termination of service;

(c) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Optionee's Shares under this paragraph, the Company shall notify the Optionee, or in case of death, his or her representative, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in subsection (b) of this paragraph for exercise of the Company's option to repurchase;

(d) The written notice to the Optionee shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten (10) days nor more than sixty (60) days from the date of the mailing of the notice, and the Optionee or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Optionee or his or her successor in interest and the Shares being purchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Optionee or his or her successor in interest; and

(e) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Optionee that the following restrictions be complied with (except as hereinafter otherwise provided) (the following restrictions, the "ROFR Restrictions"):

(a) No Shares owned by the Optionee may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the terms and conditions hereinafter set forth;

(b) Before selling or otherwise transferring all or part of the Shares, the Optionee shall give written notice of such intention to the Company, which notice shall include the name of the proposed transferee, the proposed purchase price per share, the terms of payment of such purchase price and all other matters relating to such sale or transfer, and shall be accompanied by a copy of the binding written agreement of the proposed transferee to purchase the Shares of the Optionee. Such notice shall constitute a binding offer by the Optionee to sell to the Company such number of the Shares then held by the Optionee as are proposed to be sold in the notice at the monetary price per share designated in such notice, payable on the terms offered to the Optionee by the proposed transferee (provided, however, that the Company shall not be required to meet any non-monetary terms of the proposed transfer, including, without limitation, delivery of other securities in exchange for the Shares proposed to be sold). The Company shall give written notice to the Optionee as to whether such offer has been

accepted in whole by the Company within sixty (60) days after its receipt of written notice from the Optionee. The Company may only accept such offer in whole and may not accept such offer in part. Such acceptance notice shall fix a time, location and date for the closing on such purchase ("Closing Date") which shall not be less than ten (10) nor more than sixty (60) days after the giving of the acceptance notice. The place for such closing shall be at the Company's principal office. At such closing, the Optionee shall accept payment as set forth herein and shall deliver to the Company in exchange therefor certificates for the number of Shares stated in the notice accompanied by duly executed instruments of transfer;

(c) If the Company shall fail to accept any such offer, the Optionee shall be free to sell all, but not less than all, of the Shares set forth in his or her notice to the designated transferee at the price and terms designated in the Optionee's notice, provided that (i) such sale is consummated within six (6) months after the giving of notice by the Optionee to the Company as aforesaid, and (ii) the transferee first agrees in writing to be bound by the provisions of these ROFR Restrictions so that such transferee (and all subsequent transferees) shall thereafter only be permitted to sell or transfer the Shares in accordance with the terms hereof. After the expiration of such six (6) months, the provisions of this paragraph shall again apply with respect to any proposed voluntary transfer of the Optionee's Shares;

(d) The restrictions on transfer contained in this paragraph shall not apply to (a) transfers by the Optionee to his or her spouse or children or to a trust for the benefit of his or her spouse or children, (b) transfers by the Optionee to his or her guardian or conservator, and (c) or transfers by the Optionee, in the event of his or her death, to his or her executor(s) or administrator(s) or to trustee(s) under his or her will (collectively, "Permitted Transferees"); provided however, that in any such event the Shares so transferred in the hands of each such Permitted Transferee shall remain subject to this Certificate, and each such Permitted Transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer;

(e) The provisions of this paragraph may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose; and

(f) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

In the event that the Optionee or his or her successor in interest fails to deliver the Shares to be repurchased by the Company under this Certificate, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Optionee or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Optionee to the Company and to treat the Optionee and such Shares in all respects as if delivery of such Shares had been made as required by this Certificate. The Optionee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of Company issued to the Optionee with respect to the Shares then subject to the restrictions contained in this Certificate shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Certificate. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed to the Optionee with respect to the Shares then subject to the restrictions contained in this Certificate, shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Certificate.

If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Shares then subject to the restrictions contained in this Certificate such amount and kind of securities as are issued

in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Certificate.

The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Certificate, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Certificate.

The Optionee agrees, in connection with the initial underwritten public offering of the Company's securities pursuant to a registration statement under the Securities Act of 1933, (a) not to sell, make short sale of, loan, grant any options for the purchase of, or otherwise dispose of any shares of Common Stock of the Company held by the Optionee (other than those shares included in the offering) without the prior written consent of the Company or the underwriters managing such initial underwritten public offering of the Company's securities for a period of 180 days from the effective date of such registration statement, and (b) to execute any agreement reflecting clause (a) above as may be requested by the Company or the managing underwriters at the time of such offering.

The Optionee acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Optionee any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment, consultancy or directorship of the Optionee by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

All certificates representing the Shares to be issued to the Optionee pursuant to this Certificate shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to transfer restrictions and a right of repurchase by the Company, each as set forth in an Incentive Stock Option Certificate with this Company, a copy of which Certificate is available for inspection at the offices of the Company or will be made available upon request."

Any notices required or permitted by the terms of this Certificate or the Plan shall be given by recognized overnight courier service, facsimile, or registered or certified mail, return receipt requested, addressed to the Company at its principal place of business or to the Optionee at the address set forth above or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized overnight courier service or three business days following mailing by registered or certified mail.

This Certificate, together with the Plan, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, and no other statement, representation, warranty, covenant or agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Stock Option Grant, provided, however, in any event, this Certificate shall be subject to and governed by the Plan.

IN WITNESS WHEREOF, the Company has caused this Certificate to be executed by its duly authorized officer, and the Optionee has hereunto set his or her hand, all as of the date first above written.

PTC THERAPEUTICS, INC.

By: _____
Name:
Title:

OPTIONEE

Signature: _____

Exhibit A

NOTICE OF EXERCISE OF INCENTIVE STOCK OPTION

To: PTC Therapeutics, Inc.
100 Corporate Court
Middlesex Business Center
South Plainfield, NJ 07080

Ladies and Gentlemen:

I hereby exercise my Incentive Stock Option to purchase shares (the "Shares") of the common stock, $.001 par value, of PTC Therapeutics, Inc. (the "Company"), at the exercise price of $ per share, pursuant to and subject to the terms of that certain Incentive Stock Option Certificate between the undersigned and the Company dated , 20 .

I am aware that the issuance of the Shares has not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. I understand that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by me in this Notice of Exercise.

I hereby represent and warrant that (1) I have been furnished with all information which I deem necessary to evaluate the merits and risks of the purchase of the Shares; (2) I have had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to my satisfaction; (3) I have been given the opportunity to obtain any additional information I deem necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) I have such knowledge and experience in financial and business matters that I am able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

I hereby represent and warrant that I am purchasing the Shares for my own personal account for investment and not with a view to the sale or distribution of all or any part of the Shares.

I understand that because the issuance of the Shares has not been registered under the 1933 Act, I must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

In addition to any other restrictions that may apply, I agree that I will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) a registration statement covering the Shares under the Securities Exchange Act of 1934 has been effective for at least 90 days or (2) the Company receives an opinion of my legal counsel (concurred in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

I consent to the placing of a legend on my certificate for the Shares stating that the issuance of the Shares has not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

I understand that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by me. I understand that the Company has no obligation to me to register the sale of the Shares with the SEC and has not represented to me that it will register the sale of the Shares.

I understand the terms and restrictions on the right to dispose of the Shares set forth in the PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan, as it may be amended form time to time, and the Incentive Stock Option Certificate, both of which I have carefully reviewed. I consent to the placing of a legend on my

certificate for the Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

I have considered the Federal, state and local income tax implications of the exercise of my Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

o to me; or

o to me and , as joint tenants with right of survivorship and mail the certificate to me at the following address:

My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours,

_____

Optionee (signature)

_____

Print Name

_____

Date

_____

Social Security Number

PTC Therapeutics, Inc.

NON-QUALIFIED STOCK OPTION CERTIFICATE

THIS NON-QUALIFIED STOCK OPTION CERTIFICATE (this "Certificate"), dated as of *XXXX, XXXX*, certifies that, pursuant to the PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan, as it may be amended from time to time, (the "Plan"), the Board of Directors of PTC Therapeutics, Inc. (the "Company") has granted an Option to purchase shares of Common Stock, $.001 par value per share (the "Shares"), of the Company, as follows:

Name of Optionee:	*XXXX*
Address of Optionee:	*XXXX*
	XXXX
Grant ID Number:	#*XXXX*
Number of Shares:	*XXXX*
Option Price:	US $*XXX* per share
Date of Grant:	*XXXX*
Vesting Reference Date:	*XXXX*

The Option is subject to all the terms, conditions and limitations set forth in the Plan, which is incorporated herein by reference, and to the following additional terms specified by the Board of Directors of the Company. The Optionee acknowledges receipt of a copy of the Plan. All capitalized terms used in this Certificate and not otherwise defined herein shall have the respective meanings ascribed to them in the Plan.

The Option shall vest and be exercisable as follows:

The shares under each Option shall vest ratably over a 4-year period on a quarterly basis, such that six and one-quarter percent (6 and 1/4%) of the Option becomes exercisable on each quarterly anniversary of the Vesting Reference Date.

The Option shall terminate ten (10) years from the Date of Grant or such shorter period as set forth in the Plan in the event of the Optionee's termination of service, Death or Disability.

The Option is not assignable or transferable, other than as provided in the Plan, and is a non-qualified option. Until the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934, the Optionee may exercise this Option by written notice to the Company, in substantially the form of Exhibit A attached hereto, and as provided in the Plan. Following such registration of the Shares pursuant to the Securities Exchange Act of 1934, the form of written notice for exercise of the Option shall be in a form to be approved by the Company's Board of Directors. No partial exercise of the Option may be for less than 100 full shares. In no event shall the Company be required to issue fractional shares.

The Optionee acknowledges that upon exercise of the Option the Optionee will be deemed to have taxable income measured by the difference between the then fair market value of the Shares received upon exercise and the price paid for such Shares pursuant to this Certificate. The Optionee acknowledges that any income taxes or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Optionee's responsibility.

No Shares will be issued pursuant to the exercise of this Option unless and until the Optionee pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of such exercise of this Option. The Company may withhold from the Optionee's remuneration, if any, any withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Optionee on exercise of the Option. The Optionee further agrees that, if the Company does not withhold an amount from the Optionee's remuneration sufficient to satisfy the Company's income tax withholding obligation, the Optionee will reimburse the Company on demand, in cash, for the amount under-withheld prior to the issuance of the Shares upon such exercise.

The Shares acquired by the Optionee pursuant to the exercise of the Option granted hereby shall not be transferred by the Optionee except as permitted herein. The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

In the event of the Optionee's termination of service for "cause" as defined in Article 11 of the Plan, or in the event the Administrator determines, subsequent to the Optionee's termination of service, that either prior or subsequent to Optionee's termination the Optionee engaged in conduct which would constitute "cause," then the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Certificate (including, without limitation, Shares purchased after termination of employment, consultancy or directorship, Disability or death in accordance with the Plan). In the event the Company does not exercise its option pursuant to this paragraph, the restrictions set forth in the balance of this Certificate shall not thereby lapse, and the Optionee for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this paragraph:

(a) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this paragraph shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of service;

(b) The Company's option to repurchase the Optionee's Shares in the event of termination of service shall be valid for a period of eighteen (18) months commencing with the date of such termination of service;

(c) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Optionee's Shares under this paragraph, the Company shall notify the Optionee, or in case of death, his or her representative, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in subsection (b) of this paragraph for exercise of the Company's option to repurchase;

(d) The written notice to the Optionee shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten (10) days nor more than sixty (60) days from the date of the mailing of the

notice, and the Optionee or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Optionee or his or her successor in interest and the Shares being purchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Optionee or his or her successor in interest; and

(e) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Optionee that the following restrictions be complied with (except as hereinafter otherwise provided) (the following restrictions, the "ROFR Restrictions"):

(a) No Shares owned by the Optionee may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the terms and conditions hereinafter set forth;

(b) Before selling or otherwise transferring all or part of the Shares, the Optionee shall give written notice of such intention to the Company, which notice shall include the name of the proposed transferee, the proposed purchase price per share, the terms of payment of such purchase price and all other matters relating to such sale or transfer, and shall be accompanied by a copy of the binding written agreement of the proposed transferee to purchase the Shares of the Optionee. Such notice shall constitute a binding offer by the Optionee to sell to the Company such number of the Shares then held by the Optionee as are proposed to be sold in the notice at the monetary price per share designated in such notice, payable on the terms offered to the Optionee by the proposed transferee (provided, however, that the Company shall not be required to meet any non-monetary terms of the proposed transfer, including, without limitation, delivery of other securities in exchange for the Shares proposed to be sold). The Company shall give written notice to the Optionee as to whether such offer has been accepted in whole by the Company within sixty (60) days after its receipt of written notice from the Optionee. The Company may only accept such offer in whole and may not accept such offer in part. Such acceptance notice shall fix a time, location and date for the closing on such purchase ("Closing Date") which shall not be less than

ten (10) nor more than sixty (60) days after the giving of the acceptance notice. The place for such closing shall be at the Company's principal office. At such closing, the Optionee shall accept payment as set forth herein and shall deliver to the Company in exchange therefor certificates for the number of Shares stated in the notice accompanied by duly executed instruments of transfer;

(c) If the Company shall fail to accept any such offer, the Optionee shall be free to sell all, but not less than all, of the Shares set forth in his or her notice to the designated transferee at the price and terms designated in the Optionee's notice, provided that (i) such sale is consummated within six (6) months after the giving of notice by the Optionee to the Company as aforesaid, and (ii) the transferee first agrees in writing to be bound by the provisions of these ROFR Restrictions so that such transferee (and all subsequent transferees) shall thereafter only be permitted to sell or transfer the Shares in accordance with the terms hereof. After the expiration of such six (6) months, the provisions of this paragraph shall again apply with respect to any proposed voluntary transfer of the Optionee's Shares;

(d) The restrictions on transfer contained in this paragraph shall not apply to (a) transfers by the Optionee to his or her spouse or children or to a trust for the benefit of his or her spouse or children, (b) transfers by the Optionee to his or her guardian or conservator, and (c) or transfers by the Optionee, in the event of his or her death, to his or her executor(s) or administrator(s) or to trustee(s) under his or her will (collectively, "Permitted Transferees"); provided however, that in any such event the Shares so transferred in the hands of each such Permitted Transferee shall remain subject to this Certificate, and each such Permitted Transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer;

(e) The provisions of this paragraph may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose; and

(f) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

In the event that the Optionee or his or her successor in interest fails to deliver the Shares to be repurchased by the Company under this Certificate, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Optionee or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Optionee to the Company and to treat the Optionee and such Shares in all respects as if delivery of such Shares had been made as required by this Certificate. The Optionee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of Company issued to the Optionee with respect to the Shares then subject to the restrictions contained in this Certificate shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Certificate. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed to the Optionee with respect to the Shares then subject to the restrictions contained in this Certificate, shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Certificate.

If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Shares then subject to the restrictions contained in this Certificate such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Certificate.

The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Certificate, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Certificate.

The Optionee agrees, in connection with the initial underwritten public offering of the Company's securities pursuant to a registration statement under the Securities Act of 1933, (a) not to sell, make short sale of, loan, grant any options for the purchase of, or otherwise dispose of any shares of Common Stock of the Company held by the

Optionee (other than those shares included in the offering) without the prior written consent of the Company or the underwriters managing such initial underwritten public offering of the Company's securities for a period of 180 days from the effective date of such registration statement, and (b) to execute any agreement reflecting clause (a) above as may be requested by the Company or the managing underwriters at the time of such offering.

The Optionee acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Optionee any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment, consultancy or directorship of the Optionee by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

All certificates representing the Shares to be issued to the Optionee pursuant to this Certificate shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to transfer and other restrictions, each as set forth in a Stock Option Certificate with this Company, a copy of which Certificate is available for inspection at the offices of the Company or will be made available upon request."

Any notices required or permitted by the terms of this Certificate or the Plan shall be given by recognized overnight courier service, facsimile, or registered or certified mail, return receipt requested, addressed to the Company at its principal place of business or to the Optionee at the address set forth above or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized overnight courier service or three business days following mailing by registered or certified mail.

This Certificate, together with the Plan and any other written agreement between the Company and the Optionee relevant to the subject matter hereof and executed contemporaneously herewith or hereafter, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, and no other statement, representation, warranty, covenant or agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Stock Option Grant, provided, however, in any event, this Certificate shall be subject to and governed by the Plan.

IN WITNESS WHEREOF, the Company has caused this Certificate to be executed by its duly authorized officer, and the Optionee or the Optionee's authorized representative, as the case may be, has hereunto set his or her hand, all as of the date first above written.

PTC THERAPEUTICS, INC.	**OPTIONEE:**
Name: Title:	Name: *XXXX*

Grant ID#: *XXXX*

Exhibit A

NOTICE OF EXERCISE OF NON-QUALIFIED STOCK OPTION

To: PTC Therapeutics, Inc.
100 Corporate Court
Middlesex Business Center
South Plainfield, NJ 07080

Ladies and Gentlemen:

The undersigned entity or individual (the "Optionee") hereby exercises the Non-Qualified Stock Option to purchase shares (the "Shares") of the common stock, $.001 par value, of PTC Therapeutics, Inc. (the "Company"), at the exercise price of $ per share, pursuant to and subject to the terms of that certain Non-Qualified Stock Option Certificate between the Optionee and the Company dated , 20 .

Optionee is aware that the issuance of the Shares has not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. Optionee understands that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by Optionee in this Notice of Exercise.

Optionee hereby represents and warrants that (1) Optionee has been furnished with all information which Optionee deems necessary to evaluate the merits and risks of the purchase of the Shares; (2) Optionee has had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to Optionee's satisfaction; (3) Optionee has been given the opportunity to obtain any additional information Optionee deems necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) Optionee has such knowledge and experience in financial and business matters that Optionee is able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

Optionee hereby represents and warrants that Optionee is purchasing the Shares for Optionee's own account for investment and not with a view to the sale or distribution of all or any part of the Shares.

Optionee understands that because the issuance of the Shares has not been registered under the 1933 Act, Optionee must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

In addition to any other restrictions that may apply, Optionee agrees that Optionee will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) a registration statement covering the Shares under the Securities Exchange Act of 1934 has been effective for at least 90 days or (2) the Company receives an opinion of Optionee's legal counsel (concurred in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

Optionee consents to the placing of a legend on Optionee's certificate for the Shares stating that the issuance of the Shares has not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

Optionee understands that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by Optionee. Optionee understands that the Company has no obligation to Optionee to register the sale of the Shares with the SEC and has not represented to Optionee that it will register the sale of the Shares.

Optionee understands the terms and restrictions on the right to dispose of the Shares set forth in the PTC Therapeutics, Inc. 1998 Employee, Director and Consultant Stock Option Plan, as it may be amended from time to time, and the Non-Qualified Stock Option Certificate, both of which Optionee has carefully reviewed. Optionee consents to the placing of a legend on Optionee's certificate for the Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

Optionee has considered the Federal, state and local income tax implications of the exercise of the Option and the purchase and subsequent sale of the Shares.

Optionee is paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

o to Optionee; or

o to Optionee and , as joint tenants with right of survivorship

and mail the certificate to Optionee at the following address:

Optionee's mailing address for shareholder communications, if different from the address listed above is:

Very truly yours,

_____

Optionee (signature)

_____

Print Name

_____

Date

_____

FEIN/Social Security Number

PTC THERAPEUTICS, INC.

2009 EQUITY AND LONG TERM INCENTIVE PLAN

Contents:

1. Purpose & Eligibility

A. Purpose: The purpose of this 2009 Equity and Long Term Incentive Plan (the "Plan") of PTC Therapeutics, Inc., a Delaware corporation (the "Company"), is to advance the interests of the Company's stockholders by enhancing the Company's ability to attract, retain and motivate persons who are expected to make important contributions to the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to align better the interests of such persons with those of the Company's stockholders. Except where the context otherwise requires, the term "Company" shall include any of the Company's present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Internal Revenue Code of 1986, as amended, and any regulations thereunder (the "Code") and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Board of Directors of the Company (the "Board").

B. Eligibility: All of the Company's employees, officers, directors, consultants, contractors and advisors are eligible to be granted Options (as defined in Section 4), stock appreciation rights ("SARs"), restricted stock, restricted stock units and other cash and stock based awards (each, an "Award") under the Plan. Each person who is granted an Award under the Plan is deemed a "Participant". The documentation (written, electronic or otherwise) evidencing each Award is deemed a "Notice of Award". Notwithstanding the foregoing, the Board may authorize the grant of an Award to a person not then an employee, officer, director, consultant, contractor or advisor of the Company; provided, however, that the actual grant of such Award shall be conditioned upon such person becoming eligible to become a Participant at or prior to the time of the delivery of the Notice of Award evidencing such Award.

Administration and Delegation

C. Administration by Board of Directors. The Plan will be administered by the Board. The Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Board may construe and interpret the terms of the Plan and any Notices of Award entered into under the Plan. The Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient to carry the Plan into effect and it shall be the sole and final judge of such expediency. All decisions by the Board shall be made in the Board's sole discretion and shall be final and binding on all persons having or claiming any interest in the Plan or in any Award. No director or person acting pursuant to the authority delegated by the Board shall be liable for any action or determination relating to or under the Plan made in good faith.

D. Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Board (a "Committee"). All references in the Plan to the "Board" shall mean the Board or a Committee of the Board or the officers referred to in Section 2.C to the extent that the Board's powers or authority under the Plan have been delegated to such Committee or officers.

E. Delegation to Officers. To the extent permitted by applicable law, the Board may delegate to one or more officers of the Company the power to grant Options and other Awards that constitute rights under Delaware law (subject to any limitations under the Plan) to employees or officers of the Company or any of its present or future subsidiary corporations and to exercise such other powers under the Plan as the Board may determine; provided, however, that the Board shall fix the terms of the Awards to be granted by such officers (including the exercise price of the Awards, which may include a formula by which the exercise price will be determined) and the maximum number of shares subject to such Awards that the officers may grant; and provided, further, that no officer shall be authorized to grant Awards to any "executive officer" of the Company (as defined by Rule 3b-7 under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) or to any "officer" of the Company (as defined by Rule 16a-1 under the Exchange Act). The Board may not delegate authority under this Section to grant Restricted Stock unless Delaware law then permits such delegation.

2. Stock Available for Awards

A. Number of Shares. Subject to adjustment under Section 7, Awards may be made under the Plan for up to the number of shares of common stock, par value $0.001 per share, of PTC Therapeutics, Inc. (the "Common Stock") that is equal to the sum of:

I. 800,000 shares of Common Stock; plus

II. such additional number of shares of Common Stock (up to 3,850,000 shares) as is equal to the sum of (x) the number of shares of Common Stock reserved for issuance under the Company's 1998 Employee, Director And Consultant Stock Option Plan, as amended and restated (the "1998 Plan") that remained available for grant under the 1998 Plan upon the termination of the 1998 Plan and (y) the number of shares of Common Stock subject to awards granted under the 1998 Plan which awards expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right; plus

III. an annual increase to be added on the first day of each of the Company's fiscal years during the term of the Plan beginning in fiscal year 2010 equal to the lowest of (i) 800,000 shares of Common Stock, (ii) 4% of the outstanding shares of Common Stock on such date (including for such purposes all shares of Common Stock issuable upon conversion of all then-outstanding shares of the Company's preferred stock) and (iii) an amount determined by the Board.

If any Award expires or is terminated, surrendered or canceled without having been fully exercised, is forfeited in whole or in part (including as the result of shares of Common Stock subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right), is settled in cash or otherwise results in any Common Stock not being issued, the unused Common Stock covered by such Award shall again be available for the grant of Awards under the Plan. However, in the case of Incentive Stock Options (as hereinafter defined), the foregoing provisions shall be subject to any limitations under the Code. Shares issued under the Plan may consist in whole or in part of authorized but unissued shares or treasury shares. The Board may provide that Awards be granted subject to subsequent approval by the shareholders of the Company of any amendments to this Plan; provided, however, that the actual grant of such Award shall be conditioned upon obtaining such shareholder approval prior to the time of the delivery of the Notice of Award evidencing such Award

B. Section 162(m) Per-Participant Limit. Subject to adjustment under Section 6, for Awards granted after the Common Stock is registered under the Exchange Act, the maximum number of shares of Common Stock with respect to which Awards may be granted to any Participant under the Plan shall be 400,000 per fiscal year. For purposes of the foregoing limit, the combination of an Option in tandem with a SAR shall be treated as a single Award. The per-Participant limit described in this Section 2.B shall be construed and applied consistently with Section 162(m) of the Code or any successor provision thereto, and the regulations thereunder ("Section 162(m)").

C. Substitute Awards. In connection with a merger or consolidation of an entity with the Company or the acquisition by the Company of property or stock of an entity, the Board may grant Awards in substitution for any options or other stock or stock-based awards granted by such entity or an affiliate thereof ("Substitute Awards"). Substitute Awards may be granted on such terms as the Board deems appropriate in the circumstances, notwithstanding any limitations on Awards contained in the Plan. Substitute Awards shall not count against the overall share limit set forth in Section 3.A, except as may be required by reason of Section 422 and related provisions of the Code.

3. Stock Options

A. General. The Board may grant options to purchase Common Stock (each, an "Option") and determine the number of shares of Common Stock to be covered by each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable federal or state securities laws, as it considers necessary or advisable. An Option that is not intended to be an Incentive Stock Option (as hereinafter defined) shall be a "Nonstatutory Stock Option".

B. Incentive Stock Options. An Option that the Board intends to be an "incentive stock option" as defined in Section 422 of the Code (an "Incentive Stock Option") shall only be granted to employees of the Company, any of the Company's present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Code, and any other entities the employees of which are eligible to receive Incentive Stock Options under the Code, and shall be subject to and shall be construed consistently with the requirements of Section 422 of the Code. The Company shall have no liability to a Participant, or any other party, if an Option (or any part thereof) that is intended to be an Incentive Stock Option is not an Incentive Stock Option or for any action taken by the Board, including without limitation the conversion of an Incentive Stock Option to a Nonstatutory Stock Option.

C. Exercise Price. The Board shall establish the exercise price of each Option and specify the exercise price in the Notice of Award for such Option. The exercise price shall not be less than 100% of the Fair Market Value on the date the Option is granted; provided, however, that if the Board approves the grant of an Option with an exercise price to be determined on a future date, the exercise price shall be not less than 100% of the Fair Market Value on such future date. As used in this Plan, "Fair Market Value" means the fair market value of a share of Common Stock as determined by (or in a manner approved by) the Board.

D. Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable Notice of Award; provided, however, that no Option will be granted for a term in excess of 10 years; and provided, further, that if no term is specified in the Notice of Award for an Option, then the term of such Option shall be 10 years.

E. Exercise of Option. Options may be exercised by delivery to the Company of a written notice of exercise signed by the proper person or by any other form of notice (including electronic notice) approved by the Board, together with payment in full as specified in Section 3.F for the number of shares for which the Option is exercised (which number must not result in the issuance of a fractional share). Shares of Common Stock subject to the Option will be issued by the Company following exercise as soon as practicable following exercise.

F. Payment Upon Exercise. Common Stock purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

I. in cash or by check, payable to the order of the Company;

II. when the Common Stock is registered under the Exchange Act, except as may otherwise be provided in the applicable Notice of Award, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

III. when the Common Stock is registered under the Exchange Act, to the extent provided for in the applicable Notice of Award or approved by the Board, in its sole discretion, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their Fair Market Value; provided: (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board in its discretion and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

IV. to the extent provided for in the applicable Notice of Award or approved by the Board in its sole discretion, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would receive the number of shares of Common Stock underlying the Option so exercised reduced by the number of shares of Common Stock equal to the aggregate exercise price of the Option divided by the Fair Market Value on the date of exercise; provided, however, that such provision shall only be operative in a Notice of Award for an Incentive Stock Option to the extent that the inclusion of such provision will not cause the Option to fail to qualify as an Incentive Stock Option under the applicable Code rules;

V. to the extent permitted by applicable law and provided for in the applicable Notice of Award or approved by the Board, in its sole discretion, by (i) delivery of a promissory note of the Participant to the Company on terms determined by the Board, or (ii) payment of such other lawful consideration as the Board may determine; or

VI. by any combination of the above permitted forms of payment.

Notwithstanding the foregoing, payment for Common Stock purchased upon the exercise of an Incentive Stock Option may only be made in a manner permitted by Section 422 of the Code.

G. Effect on Options of Termination of a Participant's Service for Good Cause. Except to the extent specifically provided to the contrary in the Notice of Award evidencing a particular Option, if a Participant's service (whether as an employee, director or consultant) with the Company is terminated for Good Cause prior to the time that any of such Participant's Options have been exercised in full, all such outstanding and unexercised Options as of the time the Participant is notified his or her service is terminated for Good Cause will immediately be forfeited. Furthermore, in the event that the Company determines that a Participant's discharge for Good Cause was warranted as contemplated by the last sentence of the following paragraph and such determination occurs prior to the exercise of an Option, the right to exercise any such Option shall be forfeited. The Company may suspend the ability of a Participant to exercise any Options during the time period in which the Company may determine whether discharge for Good Cause was warranted.

As used in this Plan, except as otherwise defined in a Notice of Award, or in any other agreement between the Company and a particular Participant, "Good Cause" shall mean a good faith finding by the Company that a Participant has: (i) failed to perform or was negligent in performing his or her duties diligently and such failure or negligence is not substantially cured with 30 days after such Participant's receipt of written notice from the Company of such failure or negligence, (ii) engaged in serious misconduct, including but not limited to misconduct harmful to the interests of the Company which causes economic damage to the Company (for example, misappropriation of Company funds), (iii) engaged in conduct which significantly interferes with such Participant's ability to perform his or her duties (for example, abuse of alcohol or illicit drugs, or criminal or immoral acts that damage the Company or its reputation), or (iv) a material breach by such Participant of any employment agreement, confidentiality agreement, assignment of inventions agreement, non-competition agreement or non-solicitation agreement to which such Participant is a party that is not substantially cured within 30 days after such Participant's receipt of written notice from the Company of the breach. A Participant shall be considered to have been discharged for "Good Cause" if the Company determines, within 30 days after such Participant's resignation or termination, that discharge for Good Cause was warranted and the Company provides written notice to such Participant listing the reasons for such determination no later than 45 days after such resignation or termination.

H. Effect on Options of Termination of Service for Disability. Except to the extent specifically provided to the contrary in the Notice of Award evidencing a particular Option, a Participant who ceases to be an employee, director or consultant of the Company by reason of such Participant becoming Disabled (within the meaning of Section 22(e)(3) may exercise any Option granted to such Participant:

I. To the extent that the Option has become exercisable according to the vesting period of such Option as of the date that such Participant became Disabled; and

II. To the extent of a pro rata portion through such date of disability of any additional Options that would have become exercisable on the next vesting date had the Participant not become Disabled.

The proration shall be based upon the number of days accrued in the current vesting period prior to such date of disability.

A Disabled Participant may exercise such rights only within the period ending one (1) year after the date of the Participant's termination of employment, directorship or consultancy, as the case may be, notwithstanding that the Participant might have been able to exercise the Option as to some or all of the Shares on a later date if the Participant had not become Disabled and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

The Board shall make the determination both of whether a Participant has become Disabled and the date that such Participant became Disabled (unless a procedure for such determination is set forth in another agreement between the Company and such Participant, in which case such procedure shall be used for such determination). If requested, the Participant shall be examined by a physician selected or approved by the Board, the cost of which examination shall be paid for by the Company.

I. Effect on Options of a Participant's Death. Except to the extent specifically provided to the contrary in the Notice of Award evidencing a particular Option, in the event of the death of a Participant while the Participant is an employee, director or consultant of the Company, the deceased Participant's legal representatives and/or any person or persons who acquired such Participant's rights to an Option by will or by the laws of descent and distribution (collectively, "Survivors") may exercise any outstanding Option granted to such Participant:

I. To the extent that the Option has become exercisable according to the vesting period of such Option as of the date of death; and

II. To the extent of a pro rata portion through the date of death of any additional Options that would have become exercisable on the next vesting date had such Participant not died. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of death.

If the Participant's Survivors wish to exercise the Option, they must take all necessary steps to exercise the Option within one (1) year after the date of death of such Participant, notwithstanding that the decedent might have been able to exercise the Option as to some or all of the Shares on a later date if he or she had not died and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

4. Restricted Stock; Restricted Stock Units

A. General. The Board may grant Awards entitling recipients to acquire shares of Common Stock ("Restricted Stock"), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the recipient in the event that conditions specified by the Board in the applicable Notice of Award are not satisfied prior to the end of the applicable restriction period or periods established by the Board for such Award. Instead of granting Awards for Restricted Stock, the Board may grant Awards entitling the recipient to receive shares of Common Stock or cash to be delivered at the time such Award vests ("Restricted Stock Units"). Restricted Stock and Restricted Stock Units are each referred to herein as a "Restricted Stock Award".

B. Terms and Conditions for all Restricted Stock Awards. The Board shall determine the terms and conditions of a Restricted Stock Award, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

C. Additional Provisions Relating to Restricted Stock.

I. Dividends. Participants holding shares of Restricted Stock will be entitled to all ordinary cash dividends paid with respect to such shares, unless otherwise provided by the Board. Unless otherwise provided by the Board, if any such dividends or distributions are paid in shares, or consist of a dividend or distribution to holders of Common Stock other than an ordinary cash dividend, the shares, cash or other property will be subject to the same restrictions on transferability and forfeitability as the shares of Restricted Stock with respect to which they were paid. Each dividend payment will be made no later than the end of the calendar year in which the dividends are paid to stockholders of that class of stock or, if later, the 15th day of the third month following the date the dividends are paid to stockholders of that class of stock.

II. Stock Certificates. The Company may require that any stock certificates issued in respect of shares of Restricted Stock shall be deposited in escrow by the Participant, together with a stock power endorsed in blank, with the Company (or its designee). At the expiration of the applicable restriction periods, the Company (or such designee) shall deliver the certificates no longer subject to such restrictions to the Participant or if the Participant has died, to the beneficiary designated, in a manner determined by the Board, by a Participant to receive amounts due or exercise rights of the Participant in the event of the Participant's death (the "Designated Beneficiary"). In the absence of an effective designation by a Participant, "Designated Beneficiary" shall mean the Participant's estate.

D. Additional Provisions Relating to Restricted Stock Units.

I. Settlement. Upon the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each Restricted Stock Unit, the Participant shall be entitled to receive from the Company one share of Common Stock or an amount of cash equal to the Fair Market Value of one share of Common Stock, as provided in the applicable Notice of Award. The Board may, in its discretion, provide in the applicable Notice of Award that settlement of Restricted Stock Units shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Code Section 409A.

II. Voting Rights. A Participant shall have no voting rights with respect to any Restricted Stock Units.

III. Dividend Equivalents. To the extent provided by the Board, in its sole discretion, a grant of Restricted Stock Units may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding shares of Common Stock ("Dividend Equivalents"). Dividend Equivalents may be paid currently or credited to an account for the Participants, may be settled in cash and/or shares of Common Stock and may be subject to the same restrictions on transfer and forfeitability as the Restricted Stock Units with respect to which paid, as determined by the Board in its sole discretion, subject in each case to such terms and conditions as the Board shall establish, in each case to be set forth in the applicable Notice of Award.

5. Other Stock-Based and Cash-Based Awards

A. General. Other Awards of shares of Common Stock, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, shares of Common Stock or other property, may be granted hereunder to Participants ("Other Stock-Based Awards"), including without limitation Awards entitling recipients to receive shares of Common Stock or SARs to be delivered in the future. Such Other Stock-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan. Other Stock-Based Awards may be paid in shares of Common Stock or cash, as the Board shall determine. Subject to the provisions of the Plan, the Board shall determine the terms and conditions of each Other Stock-Based or Cash-Based Award, including any purchase price applicable thereto.

B. Terms and Conditions. The Company may also grant Performance Awards (as defined below) or other Awards denominated in cash rather than shares of Common Stock ("Cash-Based Awards").

6. Adjustments for Changes in Common Stock and Certain Other Events

A. Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under this Plan, (ii) the per-Participant limit set forth in Section 3.B, (iii) the number and class of securities and exercise price per share of each outstanding Option, (iv) the share- and per-share provisions and the exercise price of each SAR, (v) the number of shares subject to and the repurchase price per share subject to each outstanding Restricted Stock Award, and (vi) the share- and per-share-related provisions and the purchase price, if any, of each outstanding Other Stock-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such stock dividend shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common Stock acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

B. Fundamental and Change in Control Events.

I. Definitions.

a. A "Fundamental Event" shall mean:

i any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled;

ii any transfer or disposition of all of the Common Stock of the Company for cash or other securities or other property pursuant to a share exchange transaction; or

iii any liquidation or dissolution of the Company.

b. A "Change in Control Event" shall mean:

i the acquisition by an individual, entity or group (within the meaning of Section 13(d)(3) or 14(d)(2) of the Exchange Act) (a "Person") of beneficial ownership of any capital stock of the Company if, after such acquisition, such Person beneficially owns (within the meaning of Rule 13d-3 promulgated under the Exchange Act) 50% or more of either (x) the aggregate number of shares of Common Stock then outstanding (the "Outstanding Company Common Stock") or (y) the combined voting power of the then-outstanding securities of the Company entitled to vote generally in the election of directors (the "Outstanding Company Voting Securities"); provided, however, that for purposes of this subsection i, the following acquisitions shall not constitute a Change in Control Event: (A) any acquisition directly from the Company (excluding an acquisition pursuant to the exercise, conversion or exchange of any security exercisable for, convertible into or exchangeable for common stock or voting securities of the Company, unless the Person exercising, converting or exchanging such security acquired such security directly from the Company or an underwriter or agent of the Company), (B) any acquisition by any employee benefit plan (or related trust) sponsored or maintained by the Company or any corporation controlled by the Company, (C) any acquisition by any corporation pursuant to a Business Combination (as defined below) which complies with clauses (x) and (y) of subsection iii of this definition or (D) any acquisition by any corporation pursuant to a Business Combination (as defined below) immediately following which either one of the following two conditions is met: (1) a majority of the Board of Directors of the Company consists of individuals who

were directors of the Company prior to such acquisition or (2) the management team of the Company consists of substantially the same group of individuals performing in similar roles to those that existed prior to such acquisition; or

ii such time as the Continuing Directors (as defined below) do not constitute a majority of the Board (or, if applicable, the Board of Directors of a successor corporation to the Company), where the term "Continuing Director" means at any date a member of the Board (x) who was a member of the Board on the date of the initial adoption of this Plan by the Board or (y) who was nominated or elected subsequent to such date by at least a majority of the directors who were Continuing Directors at the time of such nomination or election or whose election to the Board was recommended or endorsed by at least a majority of the directors who were Continuing Directors at the time of such nomination or election; provided, however, that there shall be excluded from this clause (y) any individual whose initial assumption of office occurred as a result of an actual or threatened election contest with respect to the election or removal of directors or other actual or threatened solicitation of proxies or consents, by or on behalf of a person other than the Board; or

iii the consummation of a merger, consolidation, reorganization, recapitalization or share exchange involving the Company or a sale or other disposition of all or substantially all of the assets of the Company (a "Business Combination"), unless, immediately following such Business Combination, each of the following two conditions is satisfied: (x) all or substantially all of the individuals and entities who were the beneficial owners of the Outstanding Company Common Stock and Outstanding Company Voting Securities immediately prior to such Business Combination beneficially own, directly or indirectly, more than 50% of the then-outstanding shares of common stock and the combined voting power of the then-outstanding securities entitled to vote generally in the election of directors, respectively, of the resulting or acquiring corporation in such Business Combination (which shall include, without limitation, a corporation which as a result of such transaction owns the Company or substantially all of the Company's assets either directly or through one or more subsidiaries) (such resulting or acquiring corporation is referred to herein as the "Acquiring Corporation") in substantially the same proportions as their ownership of the Outstanding Company Common Stock and Outstanding Company Voting Securities, respectively, immediately prior to

such Business Combination and (y) no Person (excluding any employee benefit plan (or related trust) maintained or sponsored by the Company or by the Acquiring Corporation) beneficially owns, directly or indirectly, 50% or more of the then-outstanding shares of common stock of the Acquiring Corporation, or of the combined voting power of the then-outstanding securities of such corporation entitled to vote generally in the election of directors (except to the extent that such ownership existed prior to the Business Combination); or the liquidation or dissolution of the Company.

c. "Good Cause" shall have the meaning set forth in Section 4.G.

d. "Good Reason" shall mean (i) a material reduction in the scope or nature of the authority, powers, functions or duties of the Participant (other than in connection with circumstances that would constitute Good Cause for termination) from and after such Fundamental Event or Change in Control Event, as the case may be, or (ii) a material reduction in the Participant's base salary from and after such Fundamental Event or Change in Control Event, as the case may be.

II. Effect on Options.

a. Fundamental Event. Upon the occurrence of a Fundamental Event (regardless of whether such event also constitutes a Change in Control Event), or the execution by the Company of any agreement with respect to a Fundamental Event (regardless of whether such event will result in a Change in Control Event), the Board shall provide that all outstanding Options shall be assumed, or equivalent options shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof); provided, however, that if such Fundamental Event also constitutes a Change in Control Event, except to the extent specifically provided to the contrary in the Notice of Award evidencing any Option or in any other agreement between a Participant and the Company, (A) one-half of the number of shares subject to the Option which were not already

vested shall be exercisable upon the occurrence of such Fundamental Event and, subject to (B) below, the remaining one-half of such number of shares shall continue to become vested in accordance with the original vesting schedule of such Option, with one-half of the number of shares that would otherwise have become vested on each subsequent vesting date in accordance with the original schedule becoming vested on each subsequent vesting date and (B) such assumed or substituted options shall become immediately exercisable in full if, on or prior to the first anniversary of the date of the consummation of the Fundamental Event, the Participant's employment with the Company or the acquiring or succeeding corporation is terminated for Good Reason by the Participant or is terminated without Good Cause by the Company or the acquiring or succeeding corporation. For purposes hereof, an Option shall be considered to be assumed if, following consummation of the Fundamental Event, the Option confers the right to purchase, for each share of Common Stock subject to the Option immediately prior to the consummation of the Fundamental Event, the consideration (whether cash, securities or other property) received as a result of the Fundamental Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Fundamental Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); provided, however, that if the consideration received as a result of the Fundamental Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise of Options to consist solely of common stock of the acquiring or succeeding corporation (or an affiliate thereof) equivalent in value (as determined by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Fundamental Event.

Notwithstanding the foregoing, if the acquiring or succeeding corporation (or an affiliate thereof) does not agree to assume, or substitute for, such Options, or in the event of a liquidation or dissolution of the Company, the Board shall, upon written notice to the Participants, provide that all then unexercised Options will become exercisable in full as of a specified time prior to the Fundamental Event and will terminate immediately prior to the consummation of such Fundamental Event, except to the extent exercised by the Participants before the consummation of such Fundamental Event; provided, however, that in the event of a Fundamental Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share of Common Stock surrendered pursuant to such Fundamental Event (the "Acquisition Price"), then the Board may instead provide that all outstanding Options shall terminate upon consummation of such Fundamental Event and that each Participant shall receive, in exchange therefor, a cash payment equal to the amount (if any) by which (A) the Acquisition Price multiplied by the number of shares of Common Stock subject to such outstanding Options (whether or not then exercisable), exceeds (B) the aggregate exercise price of such Options.

b. Change in Control Event that is not a Fundamental Event. Upon the occurrence of a Change in Control Event that does not also constitute a Fundamental Event, except to the extent specifically provided to the contrary in the Notice of Award evidencing any Option or in any other agreement between a Participant and the Company, (A) one-half of the number of shares subject to the Option which were not already vested shall be exercisable upon the occurrence of such Change in Control Event and, subject to (B) below, the remaining one-half of such number of shares shall continue to become vested in accordance with the original vesting schedule of such Option, with one-half of the number of shares that would otherwise have become vested on each subsequent vesting date in accordance with the original schedule becoming vested on each such subsequent vesting date and (B) such Option shall become immediately exercisable in full if, on or prior to the first anniversary of the date of the consummation of the Change in Control Event, the Participant's employment with the Company or the acquiring or succeeding corporation is terminated for Good Reason by the Participant or is terminated without Good Cause by the Company or the acquiring or succeeding corporation.

III. Effect on Restricted Stock Awards.

a. Fundamental Event that is not a Change in Control Event. Upon the occurrence of a Fundamental Event that is not a Change in Control Event, the repurchase and other rights of

the Company under each outstanding Restricted Stock Award shall inure to the benefit of the Company's successor and shall, unless the Board determines otherwise, apply to the cash, securities or other property which the Common Stock was converted into or exchanged for pursuant to such Fundamental Event in the same manner and to the same extent as they applied to the Common Stock subject to such Restricted Stock Award.

a. Change in Control Event. Upon the occurrence of a Change in Control Event (regardless of whether such event also constitutes a Fundamental Event), except to the extent specifically provided to the contrary in the Notice of Award evidencing any Restricted Stock Award or in any other agreement between a Participant and the Company, the vesting schedule of all Restricted Stock Awards shall be accelerated in part so that one-half of the number of shares that would otherwise have first become free from conditions or restrictions on any date after the date of the Change in Control Event shall immediately become free from conditions or restrictions. Subject to the following sentence, the remaining one-half of such number of shares shall continue to become free from conditions or restrictions in accordance with the original vesting schedule of such Restricted Stock Award, with one-half of the number of shares that would otherwise have become free from conditions or restrictions on each subsequent vesting date in accordance with the original schedule becoming free from conditions or restrictions on each subsequent vesting date. In addition, each such Restricted Stock Award shall immediately become free from all conditions or restrictions if, on or prior to the first anniversary of the date of the consummation of the Change in Control Event, the Participant's employment with the Company or the acquiring or succeeding corporation is terminated for Good Reason by the Participant or is terminated without Good Cause by the Company or the acquiring or succeeding corporation.

IV. Effect on Other Stock-Based Awards.

a. The Board may specify in the applicable Notice of Award at the time of the grant the effect of a Fundamental Event and Change in Control Event on any Other Stock-Based Award.

7. General Provisions Applicable to Awards

A. Transferability of Awards. Except as the Board may otherwise determine or provide in an Award, Awards shall not be sold, assigned, transferred, pledged or otherwise encumbered by the person to whom they are granted, either voluntarily or by operation of law, except by will or the laws of descent and distribution or pursuant to a qualified domestic relations order (or other formal division of property rights set forth in an authorized settlement agreement arising from the Participant's divorce), and, during the life of the Participant, shall be exercisable only by the Participant (or by the Participant's legal representative); provided, however, that the gratuitous transfer of the Award by the Participant to or for the benefit of any Immediate Family Member of such Participant or any trust or other entity established for the benefit of the Participant and/or an Immediate Family Member thereof shall be permitted if, with respect to such proposed transferee, the Company would be eligible to use a Registration Statement on Form S-8 for the registration of the sale of the Common Stock subject to such Award under the Securities Act; provided, further, that the Company shall not be required to recognize any such transfer until such time as the Participant and such authorized transferee shall, as a condition to such transfer, deliver to the Company a written instrument in form and substance satisfactory to the Company confirming that such transferee shall be bound by all of the terms and conditions of the Award; and, provided, further, that no option intended to be an Incentive Stock Option shall be transferable or exercisable by any person other than the Participant unless the Board shall otherwise permit. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees.

"Immediate Family Member" of any person means any child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, domestic partner and any person sharing such person's household (other than a tenant or employee).

B. Notices of Award. Each Notice of Award shall be in such form (written, electronic or otherwise) as the Board shall determine, which form need not require a signature, or other form of acknowledgement of the applicable award, by the Participant. Each Notice of Award may contain terms and conditions in addition to those set forth in the Plan.

C. Board Discretion. Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Board need not treat Participants uniformly.

D. Termination of Status. The Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights under the Award. Except as otherwise required by law or as specifically provided in a Notice of Award, military or sick leave or other bona fide leave consistent with the applicable Company policy shall not be deemed, by virtue of such leave alone, a termination of employment, provided that it does not exceed the longer of ninety (90) days or the period during which the absent Participant's reemployment rights, if any, are guaranteed by statute or by contract. Except as otherwise required by law or as specifically provided in a Notice of Award, Awards granted under the Plan shall not be affected by any change of a Participant's status within the Company, so long as such Participant remains an employee, officer, director, consultant, contractor or advisor of the Company.

E. Withholding. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under an Award. The Company may decide to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise or release from forfeiture of an Award or, if the Company so requires, at the same time as is payment of the exercise price unless the Company determines otherwise. If provided for in an Award or approved by the Board in its sole discretion, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock, including shares

retained from the Award creating the tax obligation, valued at their Fair Market Value; provided, however, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income). Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

F. Amendment of Award.

I. The Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type, changing the date of exercise or realization, and converting an Incentive Stock Option to a Nonstatutory Stock Option The Participant's consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, would not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 7 hereof.

II. The Board may, without stockholder approval, amend any outstanding Award granted under the Plan to provide an exercise price per share that is lower than the then-current exercise price per share of such outstanding Award. The Board may also, without stockholder approval, cancel any outstanding award (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan covering the same or a different number of shares of Common Stock and having an exercise price per share lower than the then-current exercise price per share of the cancelled award.

G. Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to the Plan or to remove restrictions from shares previously delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

H. Acceleration. The Board may at any time provide that any Award shall become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part, as the case may be.

I. Performance Awards.

I. Grants. When the Common Stock is registered under the Exchange Act, Restricted Stock Awards and Other Stock-Based Awards under the Plan may be made subject to the achievement of performance goals pursuant to this Section 7.I ("Performance Awards"), subject to the limit in Section 2.B on shares covered by such grants. Performance Awards can also provide for cash payments of up to $500,000 per calendar year per individual.

II. Committee. Grants of Performance Awards to any Covered Employee intended to qualify as "performance-based compensation" under Section 162(m) ("Performance-Based Compensation") shall be made only by a Committee (or subcommittee of a Committee) comprised solely of two or more directors eligible to serve on a committee making Awards qualifying as "performance-based compensation" under Section 162(m). In the case of such Awards granted to Covered Employees, references to the Board or to a Committee shall be deemed to be references to such Committee or subcommittee. "Covered Employee" shall mean any person who is, or whom the Committee, in its discretion, determines may be, a "covered employee" under Section 162(m)(3) of the Code.

III. Performance Measures. For any Award that is intended to qualify as Performance-Based Compensation, the Committee shall specify that the degree of granting, vesting and/or payout shall be subject to the achievement of one or more objective performance measures established by the Committee, which shall be based on the relative or absolute attainment of specified levels of one or any combination of the following: (a) net income, (b) earnings before or after discontinued

operations, interest, taxes, depreciation and/or amortization, (c) operating profit before or after discontinued operations and/or taxes, (d) sales, (e) sales growth, (f) earnings growth, (g) cash flow or cash position, (h) gross margins, (i) stock price, (j) market share, (k) return on sales, assets, equity or investment, (l) improvement of financial ratings, (m) achievement of balance sheet or income statement objectives; (n) total stockholder return; or (o) achievement of milestones with respect to discovery and development of therapeutic, diagnostic or prophylactic products, and may be absolute in their terms or measured against or in relationship to other companies comparably, similarly or otherwise situated. The Committee may specify that such performance measures shall be adjusted to exclude any one or more of (i) extraordinary items, (ii) gains or losses on the dispositions of discontinued operations, (iii) the cumulative effects of changes in accounting principles, (iv) the writedown of any asset, and (v) charges for restructuring and rationalization programs. Such performance measures: (i) may vary by Participant and may be different for different Awards; (ii) may be particular to a Participant or the department, branch, line of business, subsidiary or other unit in which the Participant works and may cover such period as may be specified by the Committee; and (iii) shall be set by the Committee within the time period prescribed by, and shall otherwise comply with the requirements of, Section 162(m). Awards that are not intended to qualify as Performance-Based Compensation may be based on these or such other performance measures as the Board may determine.

IV. Adjustments. Notwithstanding any provision of the Plan, with respect to any Performance Award that is intended to qualify as Performance-Based Compensation, the Committee may adjust downwards, but not upwards, the cash or number of Shares payable pursuant to such Award, and the Committee may not waive the achievement of the applicable performance measures except in the case of the death or disability of the Participant or a Change in Control Event.

V. Other. The Committee shall have the power to impose such other restrictions on Performance Awards as it may deem necessary or appropriate to ensure that such Awards satisfy all requirements for Performance-Based Compensation.

J. Agreement in Connection with Initial Public Offering. The receipt of any Award under this Plan shall be deemed to constitute the agreement by each Participant, in connection with the initial underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (1) not to (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (ii) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (i) or (ii) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address Rule 2711(f) of the Financial Industry Regulatory Authority or any similar successor provision), and (2) to execute any agreement reflecting clause (1) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the foregoing "lock-up" period.

K. Interaction with Employment Agreements. Except as otherwise expressly provided in any particular Notice of Award, in case of any conflict between the terms of a particular Notice of Award and any employment agreement to which a Participant is a party, the terms of such employment agreement shall control.

8. Miscellaneous

A. No Right to Employment or Other Status. No person shall have any claim or right to be granted an Award, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Notice of Award or in any other agreement between such Participant and the Company

B. No Rights as Stockholder. Subject to the provisions of the applicable Notice of Award, no Participant or Designated Beneficiary shall have any rights as a stockholder with respect to any shares of Common Stock to be distributed with respect to an Award until becoming the record holder of such shares.

C. Effective Date and Term of Plan. The Plan shall become effective on the date on which it is adopted by the Board. No Awards shall be granted under the Plan after the expiration of 10 years from the earlier of (i) the date on which the plan was adopted by the Board or (ii) the date the Plan was approved by the Company's stockholders, but Awards previously granted may extend beyond that date.

D. Amendment of Plan. The Board may amend, suspend or terminate the Plan or any portion thereof at any time; provided that: (i) to the extent required by Section 162(m), no Award granted to a Participant that is intended to comply with Section 162(m) after the date of such amendment shall become exercisable, realizable or vested, as applicable to such Award, unless and until such amendment shall have been approved by the Company's stockholders if required by Section 162(m) (including the vote required under Section 162(m)); and (ii) no amendment that would require stockholder approval under the rules of any securities exchange on which the Common Stock is listed may be made effective unless and until such amendment shall have been approved by the Company's stockholders. In addition, if at any time the approval of the Company's stockholders is required as to any other modification or amendment under Section 422 of the Code or any successor provision with respect to Incentive Stock Options, the Board may not effect such modification or amendment without such approval. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 12(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, provided the Board determines that such amendment does not materially and adversely affect the rights of Participants under the Plan.

E. Authorization of Sub-Plans. The Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable blue sky, securities or tax laws of various jurisdictions. The Board shall establish such sub-plans by adopting supplements to this Plan containing (i) such limitations on the Board's discretion under the Plan as the Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Board shall deem necessary or desirable. All supplements adopted by the Board shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

F. Provisions for Foreign Participants. The Board may modify Awards or Options granted to Participants who are foreign nationals or employed outside the United States or establish subplans or procedures under the Plan to recognize differences in laws, rules, regulations or customs of such foreign jurisdictions with respect to tax, securities, currency, employee benefit or other matters.

G. Compliance with Code Section 409A. The Company makes no representation or warranty and shall have no liability to the Participant or any other person if provision of or payments, compensation or other benefits under the Plan are determined to constitute non-qualified deferred compensation subject to Code Section 409A but do not satisfy the requirements under that section.

H. Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than such state.

PTC Therapeutics, Inc.

NOTICE OF AWARD FOR INCENTIVE STOCK OPTION

THIS NOTICE OF AWARD FOR INCENTIVE STOCK OPTION (this "Notice of Award"), dated as of , certifies that, pursuant to the PTC Therapeutics, Inc. 2009 Equity and Long Term Incentive Plan, as amended (the "Plan"), the Board of Directors of PTC Therapeutics, Inc. (the "Company") has granted an Option to purchase shares of Common Stock, $.001 par value per share (the "Shares"), of the Company, as follows:

Name of Optionee:
Address of Optionee:

Grant ID Number:
Number of Shares:
OPTION PRICE:
Date of Grant:
Vesting Reference Date:

The Option is subject to all the terms, conditions and limitations set forth in the Plan, which is incorporated herein by reference, and to the following additional terms specified by the Board of Directors of the Company. The Optionee acknowledges receipt of a copy of the Plan. All capitalized terms used in this Notice of Award and not otherwise defined herein shall have the respective meanings ascribed to them in the Plan.

It is intended that this Option shall be an incentive stock option as defined in Section 422 of the Code. Except as otherwise indicated by the context, the term "Optionee" as used in this Option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

The Option shall vest and be exercisable as follows:

(a) As to 25% of the original number of Shares on (the first anniversary of the Vesting Reference Date); and

(b) As to an additional 6.25% of the original number of Shares on the day following the end of each successive three-month period after , such that the first such 6.25% shall vest on , and the final such 6.25% shall vest on , upon which date all Shares under this Option shall be fully vested.

This Option shall terminate ten (10) years from the Date of Grant (the "Final Exercise Date") or such shorter period as set forth herein or in the Plan in the event of the Optionee's termination of service, Death or Disability. In the event of a termination of service other than for Death, Disability, or Good Cause, the vested portion of this Option may be exercised within three (3) months after the date the Optionee ceases to be an employee of the Company, or within the remaining term of this Option prior to the Final Exercise Date, whichever is shorter, but may not be exercised thereafter. In the event of a termination for Good Cause, the provisions of Section 3.G of the Plan shall apply to this Option. For the avoidance of doubt, Section 3.G of the Plan provides that in the event of a termination for Good Cause, this Option will immediately be forfeited and the Optionee will lose all rights to exercise the Option (whether or not vested) for Shares. In the event of a termination for Disability, the provisions of Section 3.H of the Plan shall apply to this Option. In the event of a termination for Death, the provisions of Section 3.I of the Plan shall apply to this Option.

The Option is not assignable or transferable, other than as provided in the Plan. Until the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934, as amended (the "Exchange Act"), the Optionee may exercise this Option by written notice to the Company, in substantially the form of Exhibit A attached hereto, and as provided in the Plan, together with payment in full in the manner provided in the Plan. Following such registration of the Shares pursuant to the Exchange Act, the form of written notice for exercise of the Option shall be in a form to be approved by the Company's board of directors. No partial exercise of the Option may be for less than 100 full shares. In no event shall the Company be required to issue fractional shares.

The Optionee acknowledges that any income taxes or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Optionee's responsibility. If the Optionee disposes of Shares acquired upon exercise of this Option within two years from the Grant Date or one year after such Shares were acquired pursuant to exercise of this Option, the Optionee shall notify the Company in writing of such disposition.

No Shares will be issued pursuant to the exercise of this Option unless and until the Optionee pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of such exercise of this Option. The Company may withhold from the Optionee's remuneration, if any, any withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Optionee on exercise of the Option; provided that if Shares are withheld only the statutory minimum amount of withholding will be made. The Optionee further agrees that, if the Company does not withhold an amount from the Optionee's remuneration sufficient to satisfy the Company's income tax withholding obligation, the Optionee will reimburse the Company on demand, in cash, for the amount under-withheld prior to the issuance of the Shares upon such exercise.

The Shares acquired by the Optionee pursuant to the exercise of the Option granted hereby shall not be transferred by the Optionee except as permitted herein or by the Plan. The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Exchange Act.

In the event of the Optionee's termination of service for "Good Cause" as defined in Section 3.G of the Plan, or in the event the Company determines, subsequent to the Optionee's termination of service, that either prior or subsequent to Optionee's termination the Optionee engaged in conduct which would constitute "Good Cause," then the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Notice of Award

(including, without limitation, Shares purchased after termination of employment, consultancy or directorship, Disability or death in accordance with the Plan). In the event the Company does not exercise its option pursuant to this paragraph, the restrictions set forth in the balance of this Notice of Award shall not thereby lapse, and the Optionee for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this paragraph:

(a) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this paragraph shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of service;

(b) The Company's option to repurchase the Optionee's Shares in the event of termination of service shall be valid for a period of eighteen (18) months commencing with the date of such termination of service;

(c) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Optionee's Shares under this paragraph, the Company shall notify the Optionee, or in case of death, his or her representative, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in subsection (b) of this paragraph for exercise of the Company's option to repurchase;

(d) The written notice to the Optionee shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten (10) days nor more than sixty (60) days from the date of the mailing of the notice, and the Optionee or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Optionee or his or her successor in interest and the Shares being purchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Optionee or his or her successor in interest; and

(e) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Exchange Act.

It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Optionee that the following restrictions be complied with (except as hereinafter otherwise provided) (the following restrictions, the "Transfer Restrictions"):

(a) No Shares owned by the Optionee may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the following transactions:

(i) any transfer of Shares to or for the benefit of any Immediate Family Member (as defined in the Plan) of the Optionee, any trust in which any such Immediate Family Members or such Optionee have more than 50% of the beneficial interests, a foundation in which any such Immediate Family Members or such Optionee control the management of assets or any other entity in which any such Immediate Family Members or such Optionee own more than 50% of the voting interests; provided, however, that Shares transferred pursuant to this clause (i) shall remain subject to Transfer Restrictions set forth in this Notice of Award and the transferee of such Shares shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of such Transfer Restrictions;

(ii) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the "Securities Act"); and

(iii) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation).

(b) These Transfer Restrictions may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose; and

(c) These Transfer Restrictions shall terminate upon the earlier of the following events:

(i) the closing of the sale of shares of the Company's Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(ii) the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company's voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 50% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction).

In the event that the Optionee or his or her successor in interest fails to deliver any Shares to be repurchased by the Company under this Notice of Award, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Optionee or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Optionee to the Company and to treat the Optionee and such Shares in all respects as if delivery of such Shares had been made as required by this Notice of Award. The Optionee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of Company issued to the Optionee with respect to the Shares then subject to the restrictions contained in this Notice of Award shall be added to the Shares subject to the Company's right to repurchase pursuant to this Notice of Award. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed to the Optionee with respect to the Shares then subject to the restrictions contained in this Notice of Award, shall be added to the Shares subject to the Company's right to repurchase pursuant to this Notice of Award.

If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Shares then subject to the restrictions contained in this Notice of Award such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Notice of Award.

The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Notice of Award, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Notice of Award.

The Optionee acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Optionee any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment, consultancy or directorship of the Optionee by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

All certificates representing the Shares to be issued to the Optionee pursuant to this Notice of Award shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to transfer restrictions and a right of repurchase by the Company, each as set forth in a Notice of Award for Incentive Stock Option with this Company, a copy of which Notice of Award is available for inspection at the offices of the Company or will be made available upon request."

Any notices required or permitted by the terms of this Notice of Award or the Plan shall be given by recognized overnight courier service, facsimile, or registered or certified mail, return receipt requested, addressed to the Company at its principal place of business or to the Optionee at the address set forth above or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized overnight courier service or three business days following mailing by registered or certified mail.

This Notice of Award, together with the Plan, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, and no other statement, representation, warranty, covenant or agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Stock Option Grant, provided, however, in any event, this Notice of Award shall be subject to and governed by the Plan.

Exhibit A

NOTICE OF EXERCISE OF INCENTIVE STOCK OPTION

To: PTC Therapeutics, Inc.
100 Corporate Court
Middlesex Business Center
South Plainfield, NJ 07080

Ladies and Gentlemen:

I hereby exercise my Incentive Stock Option to purchase shares (the "Shares") of the common stock, $.001 par value, of PTC Therapeutics, Inc. (the "Company"), at the exercise price of $ per share, pursuant to and subject to the terms of that certain Notice of Award for Incentive Stock Option between the undersigned and the Company dated , 20 .

I am aware that the issuance of the Shares has not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. I understand that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by me in this Notice of Exercise.

I hereby represent and warrant that (1) I have been furnished with all information which I deem necessary to evaluate the merits and risks of the purchase of the Shares; (2) I have had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to my satisfaction; (3) I have been given the opportunity to obtain any additional information I deem necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) I have such knowledge and experience in financial and business matters that I am able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

I hereby represent and warrant that I am purchasing the Shares for my own personal account for investment and not with a view to the sale or distribution of all or any part of the Shares.

I understand that because the issuance of the Shares has not been registered under the 1933 Act, I must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

In addition to any other restrictions that may apply, I agree that I will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) a registration statement covering the Shares under the Securities Exchange Act of 1934 has been effective for at least 90 days or (2) the Company receives an opinion of my legal counsel (concurred in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

I consent to the placing of a legend on my certificate for the Shares stating that the issuance of the Shares has not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

I understand that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by me. I understand that the Company has no obligation to me to register the sale of the Shares with the SEC and has not represented to me that it will register the sale of the Shares.

I understand the terms and restrictions on the right to dispose of the Shares set forth in the PTC Therapeutics, Inc. 2009 Equity and Long Term Incentive Plan, as it may be amended form time to time, and the Notice of Exercise for Incentive Stock Option, both of which I have carefully reviewed. I consent to the placing of a legend on my

certificate for the Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

I have considered the Federal, state and local income tax implications of the exercise of my Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

o to me; or

o to me and , as joint tenants with right of survivorship

and mail the certificate to me at the following address:

My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours,

_____

Optionee (signature)

_____

Print Name

_____

Date

_____

Social Security Number

PTC Therapeutics, Inc.

NOTICE OF AWARD FOR NONSTATUTORY STOCK OPTION

THIS NOTICE OF AWARD FOR NONSTATUTORY STOCK OPTION (this "Notice of Award"), dated as of the *XX* day of *XXXX, XXXX*, certifies that, pursuant to the PTC Therapeutics, Inc. 2009 Equity and Long Term Incentive Plan, as amended (the "Plan"), the Board of Directors of PTC Therapeutics, Inc. (the "Company") has granted an Option to purchase shares of Common Stock, $.001 par value per share (the "Shares"), of the Company, as follows:

Name of Optionee:	*XXXX*
Address of Optionee:	*XXXX*
	XXXX
Grant ID Number:	#*XXXX*
Number of Shares:	*XXXX*
Option Price:	US $*XXX* per share
Date of Grant:	*XXXX*
Vesting Reference Date:	*XXXX*

The Option is subject to all the terms, conditions and limitations set forth in the Plan, which is incorporated herein by reference, and to the following additional terms specified by the Board of Directors of the Company. The Optionee acknowledges receipt of a copy of the Plan. All capitalized terms used in this Notice of Award and not otherwise defined herein shall have the respective meanings ascribed to them in the Plan.

It is intended that this Option shall not be an incentive stock option as defined in Section 422 of the Code. Except as otherwise indicated by the context, the term "Optionee" as used in this Option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

The Option shall vest and be exercisable as follows:

(a) The Shares shall vest ratably over a three-year period beginning on the Vesting Reference Date, on a quarterly basis, such that eight and one-third percent (8.33%) of the Shares shall become exercisable on each quarterly anniversary of the Vesting Reference Date, provided, however, that in the event of the Optionee's termination of service, vesting of the Option shall cease as of the date of termination and Optionee shall be entitled to exercise only the vested portion of the Option up to and until the expiration of the term of the Option.

This Option shall terminate ten (10) years from the Date of Grant (the "Final Exercise Date") or such shorter period as set forth in the Plan in the event of the Optionee's termination of service, Death or Disability. In the event of a termination for Good Cause, the provisions of Section 4.G of the Plan shall apply to this Option. For the avoidance of doubt, Section 4.G of the Plan provides that in the event of a termination for Good Cause, this Option will immediately be forfeited and the Optionee will lose all rights to exercise the Option (whether or not vested) for Shares. In the event of a termination for Disability, the provisions of Section 4.H of the Plan shall apply to this Option. In the event of a termination for Death, the provisions of Section 4.I of the Plan shall apply to this Option. In the event of a termination of service for other than Good Cause, Death, or Disability, the portion of this Option that has become exercisable according to the foregoing vesting schedule as of the date of termination of service may be exercised through the Final Exercise Date.

The provisions of Section 7.B of the Plan with respect to the effect on Options of a Fundamental Event or Change of Control Event shall apply to this Option; provided, however, that the Change of Control Event shall be deemed a termination of service by the Company for other than Good Cause (and, for clarity, an election by the Optionee to terminate his or her service upon the occurrence of such Change of Control Event shall be deemed a termination for Good Reason).

The Option is not assignable or transferable, other than as provided in the Plan. Until the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934, as amended (the "Exchange Act"), the Optionee may exercise this Option by written notice to the Company, in substantially the form of Exhibit A attached hereto, and as provided in the Plan, together with payment in full in the manner provided in the Plan. Following such

registration of the Shares pursuant to the Exchange Act, the form of written notice for exercise of the Option shall be in a form to be approved by the Company's board of directors. No partial exercise of the Option may be for less than 100 full shares. In no event shall the Company be required to issue fractional shares.

The Optionee acknowledges that upon exercise of the Option the Optionee will be deemed to have taxable income measured by the difference between the then fair market value of the Shares received upon exercise and the price paid for such Shares pursuant to this Certificate. The Optionee acknowledges that any income taxes or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Optionee's responsibility.

No Shares will be issued pursuant to the exercise of this Option unless and until the Optionee pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of such exercise of this Option. The Company may withhold from the Optionee's remuneration, if any, any withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Optionee on exercise of the Option; provided that if Shares are withheld only the statutory minimum amount of withholding will be made. The Optionee further agrees that, if the Company does not withhold an amount from the Optionee's remuneration sufficient to satisfy the Company's income tax withholding obligation, the Optionee will reimburse the Company on demand, in cash, for the amount under-withheld prior to the issuance of the Shares upon such exercise.

The Shares acquired by the Optionee pursuant to the exercise of the Option granted hereby shall not be transferred by the Optionee except as permitted herein or by the Plan. The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Exchange Act.

In the event of the Optionee's termination of service for "Good Cause" as defined in Section 4.G of the Plan, or in the event the Company determines, subsequent to the Optionee's termination of service, that either prior or subsequent to Optionee's termination the Optionee engaged in conduct which would constitute "Good Cause," then the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Notice of Award (including, without limitation, Shares purchased after termination of employment, consultancy or directorship, Disability or death in accordance with the Plan). In the event the Company does not exercise its option pursuant to this paragraph, the restrictions set forth in the balance of this Notice of Award shall not thereby lapse, and the Optionee for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this paragraph:

(a) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this paragraph shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of service;

(b) The Company's option to repurchase the Optionee's Shares in the event of termination of service shall be valid for a period of eighteen (18) months commencing with the date of such termination of service;

(c) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Optionee's Shares under this paragraph, the Company shall notify the Optionee, or in case of death, his or her representative, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in subsection (b) of this paragraph for exercise of the Company's option to repurchase;

(d) The written notice to the Optionee shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten (10) days nor more than sixty (60) days from the date of the mailing of the notice, and the Optionee or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Optionee or his or her successor in interest and the Shares being purchased,

duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Optionee or his or her successor in interest; and

(e) The provisions of this paragraph shall terminate upon the effective date of the registration of the Shares pursuant to the Exchange Act.

It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Optionee that the following restrictions be complied with (except as hereinafter otherwise provided) (the following restrictions, the "Transfer Restrictions"):

(a) No Shares owned by the Optionee may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the following transactions:

(i) any transfer of Shares to or for the benefit of any Immediate Family Member (as defined in the Plan) of the Optionee, any trust in which any such Immediate Family Members or such Optionee have more than 50% of the beneficial interests, a foundation in which any such Immediate Family Members or such Optionee control the management of assets or any other entity in which any such Immediate Family Members or such Optionee own more than 50% of the voting interests; provided, however, that Shares transferred pursuant to this clause (i) shall remain subject to Transfer Restrictions set forth in this Notice of Award and the transferee of such Shares shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of such Transfer Restrictions;

(ii) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the "Securities Act"); and

(iii) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation).

(b) These Transfer Restrictions may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose; and

(c) These Transfer Restrictions shall terminate upon the earlier of the following events:

(i) the closing of the sale of shares of the Company's Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(ii) the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company's voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 50% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction).

In the event that the Optionee or his or her successor in interest fails to deliver any Shares to be repurchased by the Company under this Notice of Award, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Optionee or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Optionee to the Company and to treat the Optionee and such Shares in all respects as if delivery of such Shares had been made as required by this Notice of Award. The Optionee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of Company issued to the Optionee with respect to the Shares then subject to the restrictions contained

in this Notice of Award shall be added to the Shares subject to the Company's right to repurchase pursuant to this Notice of Award. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed to the Optionee with respect to the Shares then subject to the restrictions contained in this Notice of Award, shall be added to the Shares subject to the Company's right to repurchase pursuant to this Notice of Award.

If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Shares then subject to the restrictions contained in this Notice of Award such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Notice of Award.

The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Notice of Award, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Notice of Award.

The Optionee acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Optionee any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment, consultancy or directorship of the Optionee by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

All certificates representing the Shares to be issued to the Optionee pursuant to this Notice of Award shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to transfer restrictions and a right of repurchase by the Company, each as set forth in a Notice of Award for Nonstatutory Stock Option with this Company, a copy of which Notice of Award is available for inspection at the offices of the Company or will be made available upon request."

Any notices required or permitted by the terms of this Notice of Award or the Plan shall be given by recognized overnight courier service, facsimile, or registered or certified mail, return receipt requested, addressed to the Company at its principal place of business or to the Optionee at the address set forth above or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized overnight courier service or three business days following mailing by registered or certified mail.

This Notice of Award, together with the Plan and any other written agreement between the Company and the Optionee relevant to the subject matter hereof and executed contemporaneously herewith or hereafter, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, and no other statement, representation, warranty, covenant or agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Stock Option Grant, provided, however, in any event, this Notice of Award shall be subject to and governed by the Plan.

Exhibit A

NOTICE OF EXERCISE OF NONSTATUTORY STOCK OPTION

To: PTC Therapeutics, Inc.
100 Corporate Court
Middlesex Business Center
South Plainfield, NJ 07080

Ladies and Gentlemen:

I hereby exercise my Nonstatutory Stock Option to purchase shares (the "Shares") of the common stock, $.001 par value, of PTC Therapeutics, Inc. (the "Company"), at the exercise price of $ per share, pursuant to and subject to the terms of that certain Notice of Award for Nonstatutory Stock Option between the undersigned and the Company dated , 20 .

I am aware that the issuance of the Shares has not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. I understand that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by me in this Notice of Exercise.

I hereby represent and warrant that (1) I have been furnished with all information which I deem necessary to evaluate the merits and risks of the purchase of the Shares; (2) I have had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to my satisfaction; (3) I have been given the opportunity to obtain any additional information I deem necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) I have such knowledge and experience in financial and business matters that I am able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

I hereby represent and warrant that I am purchasing the Shares for my own personal account for investment and not with a view to the sale or distribution of all or any part of the Shares.

I understand that because the issuance of the Shares has not been registered under the 1933 Act, I must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

In addition to any other restrictions that may apply, I agree that I will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) a registration statement covering the Shares under the Securities Exchange Act of 1934 has been effective for at least 90 days or (2) the Company receives an opinion of my legal counsel (concurred in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

I consent to the placing of a legend on my certificate for the Shares stating that the issuance of the Shares has not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

I understand that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by me. I understand that the Company has no obligation to me to register the sale of the Shares with the SEC and has not represented to me that it will register the sale of the Shares.

I understand the terms and restrictions on the right to dispose of the Shares set forth in the PTC Therapeutics, Inc. 2009 Equity Incentive Plan, as it may be amended form time to time, and the Notice of Exercise for Nonstatutory Stock Option, both of which I have carefully reviewed. I consent to the placing of a legend on my certificate for the

Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

I have considered the Federal, state and local income tax implications of the exercise of my Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

o to me; or

o to me and , as joint tenants with right of survivorship

and mail the certificate to me at the following address:

My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours,

_____
Optionee (signature)

_____
Print Name

_____
Date

_____
Social Security Number

PTC THERAPEUTICS, INC.

2013 STOCK INCENTIVE PLAN

1. Purpose

The purpose of this 2013 Stock Incentive Plan (the "***Plan***") of PTC Therapeutics, Inc., a Delaware corporation (the "***Company***"), is to advance the interests of the Company's stockholders by enhancing the Company's ability to attract, retain and motivate persons who are expected to make important contributions to the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to better align the interests of such persons with those of the Company's stockholders. Except where the context otherwise requires, the term "***Company***" shall include any of the Company's present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Internal Revenue Code of 1986, as amended, and any regulations thereunder (the "***Code***") and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Board of Directors of the Company (the "***Board***"); *provided*, *however*, that such other business ventures shall be limited to entities that, where required by Section 409A of the Code, are eligible issuers of service recipient stock (as defined in Treas. Reg. Section 1.409A-1(b)(5)(iii)(E), or applicable successor regulation).

2. Eligibility

All of the Company's employees, officers and directors, as well as consultants and advisors to the Company (as such terms consultants and advisors are defined and interpreted for purposes of Rule 701 under the Securities Act of 1933, as amended (the "***Securities Act***") (or any successor rule)) are eligible to be granted Awards under the Plan. Each person who is granted an Award under the Plan is deemed a "***Participant***." "***Award***" means Options (as defined in Section 5), SARs (as defined in Section 6), Restricted Stock (as defined in Section 7), Restricted Stock Units (as defined in Section 7) and Other Stock-Based Awards (as defined in Section 8).

3. Administration and Delegation

(a) Administration by the Board. The Plan will be administered by the Board. The Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Board may construe and interpret the terms of the Plan and any Award agreements entered into under the Plan. The Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient to carry the Plan into effect and it shall be the sole and final judge of such expediency. All decisions by the Board shall be made in the Board's sole discretion and shall be final and binding on all persons having or claiming any interest in the Plan or in any Award.

(b) Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Board (each, a "***Committee***"). All references in the Plan to the "***Board***"

shall mean the Board or a Committee of the Board to the extent that the Board's powers or authority under the Plan have been delegated to such Committee.

4. Stock Available for Awards

(a) Number of Shares. Subject to adjustment under Section 9, Awards may be made under the Plan for up to 739,937 shares of common stock, $0.001 par value per share, of the Company (the "***Common Stock***"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b)). If any Award expires or is terminated, surrendered or canceled without having been fully exercised, is forfeited in whole or in part (including as the result of shares of Common Stock subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right), or results in any Common Stock not being issued, the unused Common Stock covered by such Award shall again be available for the grant of Awards under the Plan. Further, shares of Common Stock tendered to the Company by a Participant to exercise an Award or to satisfy tax withholding obligations arising with respect to an Award shall be added to the number of shares of Common Stock available for the grant of Awards under the Plan. However, in the case of Incentive Stock Options, the two immediately preceding sentences shall be subject to any limitations under the Code. Shares issued under the Plan may consist in whole or in part of authorized but unissued shares or treasury shares.

(b) Substitute Awards. In connection with a merger or consolidation of an entity with the Company or the acquisition by the Company of property or stock of an entity, the Board may grant Awards in substitution for any options or other stock or stock-based awards granted by such entity or an affiliate thereof. Substitute Awards may be granted on such terms as the Board deems appropriate in the circumstances, notwithstanding any limitations on Awards contained in the Plan. Substitute Awards shall not count against the overall share limit set forth in Section 4(a), except as may be required by reason of Section 422 and related provisions of the Code.

5. Stock Options

(a) General. The Board may grant options to purchase Common Stock (each, an "***Option***") and determine the number of shares of Common Stock to be covered by each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable federal or state securities laws, as it considers necessary or advisable.

(b) Incentive Stock Options. The Board may grant Incentive Stock Options (defined below) solely to the extent that shareholder approval of this Plan is obtained within 12 months of the date that the Board adopts this Plan. An Option that the Board intends to be an "incentive stock option" as defined in Section 422 of the Code (an "***Incentive Stock Option***") shall only be granted to employees of PTC Therapeutics, Inc., any of PTC Therapeutics, Inc.'s present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Code, and any other entities the employees of which are eligible to receive Incentive Stock Options under the Code, and shall be subject to and shall be construed consistently with the requirements of Section 422 of the Code. An Option that is not intended to be an Incentive Stock Option shall be designated a "***Nonstatutory Stock Option***." The Company shall have no liability to a Participant,

or any other party, if an Option (or any part thereof) that is intended to be an Incentive Stock Option is not an Incentive Stock Option or if the Company converts an Incentive Stock Option to a Nonstatutory Stock Option.

(c) Exercise Price. The Board shall establish the exercise price of each Option and specify the exercise price in the applicable Option agreement. The exercise price shall be not less than 100% of the fair market value per share of Common Stock, as determined by (or in a manner approved by) the Board ("***Fair Market Value***"), on the date the Option is granted. "**Fair Market Value**" of a share of Common Stock for purposes of the Plan will be determined as follows:

(1) if the Common Stock is not publicly traded, the Board will determine the Fair Market Value for purposes of the Plan using any measure of value it determines to be appropriate (including, as it considers appropriate, relying on appraisals) in a manner consistent with the valuation principles under Code Section 409A, except as the Board may expressly determine otherwise;

(2) if the Common Stock trades on a national securities exchange, the closing sale price (for the primary trading session) on the date of grant; or

(3) if the Common Stock does not trade on any such exchange, the average of the closing bid and asked prices as reported by an authorized OTCBB market data vendor as listed on the OTCBB website (otcbb.com) on the date of grant.

For any date that is not a trading day, the Fair Market Value of a share of Common Stock for such date will be determined by using the closing sale price or average of the bid and asked prices, as appropriate, for the immediately preceding trading day and with the timing in the formulas above adjusted accordingly. The Board can substitute a particular time of day or other measure of "closing sale price" or "bid and asked prices" if appropriate because of exchange or market procedures or can, in its sole discretion, use weighted averages either on a daily basis or such longer period as complies with Code Section 409A.

The Board has sole discretion to determine the Fair Market Value for purposes of the Plan, and all Awards are conditioned on the participants' agreement that the Administrator's determination is conclusive and binding even though others might make a different determination.

(d) Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable option agreement; *provided*, *however*, that no Option will be granted with a term in excess of 10 years.

(e) Exercise of Options. Options may be exercised by delivery to the Company of a notice of exercise in a form of notice (which may be electronic) approved by the Company, together with payment in full (in a manner specified in Section 5(f)) of the exercise price for the number of shares for which the Option is exercised. Shares of Common Stock subject to the Option will be delivered by the Company as soon as practicable following exercise.

(f) Payment Upon Exercise. Common Stock purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

(1) in cash or by check, payable to the order of the Company;

(2) when the Common Stock is registered under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), except as may otherwise be provided in the applicable Option agreement or approved by the Board, in its sole discretion, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) when the Common Stock is registered under the Exchange Act and to the extent provided for in the applicable Option agreement or approved by the Board, in its sole discretion, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their Fair Market Value, *provided* (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board in its discretion and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent provided for in the applicable Nonstatutory Stock Option agreement or approved by the Board in its sole discretion, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would pay the exercise price for the portion of the Option being exercised by cancelling a portion of the Option for such number of shares as is equal to the exercise price divided by the Fair Market Value per Share on the date of exercise.

(5) to the extent permitted by applicable law and provided for in the applicable Option agreement or approved by the Board, in its sole discretion, by (i) delivery of a promissory note of the Participant to the Company on terms determined by the Board, or (ii) payment of such other lawful consideration as the Board may determine; or

(6) by any combination of the above permitted forms of payment.

6. Stock Appreciation Rights

(a) General. The Board may grant Awards consisting of stock appreciation rights ("***SARs***") entitling the holder, upon exercise, to receive an amount of Common Stock or cash or a combination thereof (such form to be determined by the Board) determined by reference to appreciation, from and after the date of grant, in the Fair Market Value of a share of Common Stock over the measurement price established pursuant to Section 6(b). The date as of which such appreciation is determined shall be the exercise date.

(b) Measurement Price. The Board shall establish the measurement price of each SAR and specify it in the applicable SAR agreement. The measurement price shall not be less than 100% of the Fair Market Value on the date the SAR is granted.

(c) Duration of SARs. Each SAR shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable SAR agreement; *provided, however*, that no SAR will be granted with a term in excess of 10 years.

(d) Exercise of SARs. SARs may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with any other documents required by the Board.

7. Restricted Stock; Restricted Stock Units

(a) General. The Board may grant Awards entitling recipients to acquire shares of Common Stock ("***Restricted Stock***"), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the recipient in the event that conditions specified by the Board in the applicable Award are not satisfied prior to the end of the applicable restriction period or periods established by the Board for such Award. The Board may also grant Awards entitling the recipient to receive shares of Common Stock or cash to be delivered at the time such Award vests ("***Restricted Stock Units***") (Restricted Stock and Restricted Stock Units are each referred to herein as a "***Restricted Stock Award***").

(b) Terms and Conditions for All Restricted Stock Awards. The Board shall determine the terms and conditions of a Restricted Stock Award, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

(c) Additional Provisions Relating to Restricted Stock.

(1) Dividends. Unless otherwise provided in the applicable Award agreement, any dividends (whether paid in cash, stock or property) declared and paid by the Company with respect to shares of Restricted Stock ("***Accrued Dividends***") shall be paid to the Participant only if and when such shares become free from the restrictions on transferability and forfeitability that apply to such shares. Each payment of Accrued Dividends will be made no later than the end of the calendar year in which the dividends are paid to stockholders of that class of stock or, if later, the 15th day of the third month following the lapsing of the restrictions on transferability and the forfeitability provisions applicable to the underlying shares of Restricted Stock.

(2) Stock Certificates. The Company may require that any stock certificates issued in respect of shares of Restricted Stock, as well as dividends or distributions paid on such Restricted Stock, shall be deposited in escrow by the Participant, together with a stock power endorsed in blank, with the Company (or its designee). At the expiration of the applicable restriction periods, the Company (or such designee) shall deliver the certificates no longer subject to such restrictions to the Participant or if the Participant has died, to his or her Designated Beneficiary. "***Designated Beneficiary***" means (i) the beneficiary designated, in a manner determined by the Board, by a Participant to receive amounts due or exercise rights of

the Participant in the event of the Participant's death or (ii) in the absence of an effective designation by a Participant, "***Designated Beneficiary***" the Participant's estate.

(d) Additional Provisions Relating to Restricted Stock Units.

(1) Settlement. Upon the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each Restricted Stock Unit, the Participant shall be entitled to receive from the Company one share of Common Stock or (if so provided in the applicable Award agreement) an amount of cash equal to the Fair Market Value of one share of Common Stock. The Board may, in its discretion, provide that settlement of Restricted Stock Units shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Section 409A of the Code.

(2) Voting Rights. A Participant shall have no voting rights with respect to any Restricted Stock Units.

(3) Dividend Equivalents. The Award agreement for Restricted Stock Units may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding shares of Common Stock ("***Dividend Equivalents***"). Dividend Equivalents may be paid currently or credited to an account for the Participants, may be settled in cash and/or shares of Common Stock and may be subject to the same restrictions on transfer and forfeitability as the Restricted Stock Units with respect to which paid, in each case to the extent provided in the applicable Award agreement.

8. Other Stock-Based Awards

(a) General. Other Awards of shares of Common Stock, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, shares of Common Stock or other property, may be granted hereunder to Participants ("***Other Stock-Based Awards***"). Such Other Stock-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan or as payment in lieu of compensation to which a Participant is otherwise entitled. Other Stock-Based Awards may be paid in shares of Common Stock or cash, as the Board shall determine.

(b) Terms and Conditions. Subject to the provisions of the Plan, the Board shall determine the terms and conditions of each Other Stock-Based Award, including any purchase price applicable thereto.

9. Adjustments for Changes in Common Stock and Certain Other Events

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under the Plan, (ii) the number and class of securities and exercise price per share of each outstanding Option, (iii) the share and per-share provisions and the measurement price of each outstanding SAR, (iv) the number of shares subject to and the repurchase price per share subject to each outstanding Restricted Stock Award and (v) the share and per-share-related

provisions and the purchase price, if any, of each outstanding Other Stock-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such stock dividend shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common Stock acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

(b) Reorganization Events.

(1) Definition. A "***Reorganization Event***" shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (b) any transfer or disposition of all of the Common Stock of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company.

(2) Consequences of a Reorganization Event on Awards Other than Restricted Stock.

(i) In connection with a Reorganization Event, the Board may take any one or more of the following actions as to all or any (or any portion of) outstanding Awards other than Restricted Stock on such terms as the Board determines (except to the extent specifically provided otherwise in an applicable Award agreement or another agreement between the Company and the Participant): (i) provide that such Awards shall be assumed, or substantially equivalent Awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to a Participant, provide that all of the Participant's unexercised Awards will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant (to the extent then exercisable) within a specified period following the date of such notice, (iii) provide that outstanding Awards shall become exercisable, realizable, or deliverable, or restrictions applicable to an Award shall lapse, in whole or in part prior to or upon such Reorganization Event, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the "***Acquisition Price***"), make or provide for a cash payment to Participants with respect to each Award held by a Participant equal to (A) the number of shares of Common Stock subject to the vested portion of the Award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such Reorganization Event) multiplied by (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise, measurement or purchase price of such Award and any applicable tax withholdings, in exchange for the termination of such Award, (v) provide that, in connection with a liquidation or dissolution of the Company, Awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination of the

foregoing. In taking any of the actions permitted under this Section 9(b)(2), the Board shall not be obligated by the Plan to treat all Awards, all Awards held by a Participant, or all Awards of the same type, identically.

(ii) Notwithstanding the terms of Section 9(b)(2)(i), in the case of outstanding Restricted Stock Units that are subject to Section 409A of the Code: (i) if the applicable Restricted Stock Unit agreement provides that the Restricted Stock Units shall be settled upon a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i), and the Reorganization Event constitutes such a "change in control event", then no assumption or substitution shall be permitted pursuant to Section 9(b)(2)(i)(i) and the Restricted Stock Units shall instead be settled in accordance with the terms of the applicable Restricted Stock Unit agreement; and (ii) the Board may only undertake the actions set forth in clauses (iii), (iv) or (v) of Section 9(b)(2)(i) if the Reorganization Event constitutes a "change in control event" as defined under Treasury Regulation Section 1.409A-3(i)(5)(i) and such action is permitted or required by Section 409A of the Code; if the Reorganization Event is not a "change in control event" as so defined or such action is not permitted or required by Section 409A of the Code, and the acquiring or succeeding corporation does not assume or substitute the Restricted Stock Units pursuant to clause (i) of Section 9(b)(2)(i), then the unvested Restricted Stock Units shall terminate immediately prior to the consummation of the Reorganization Event without any payment in exchange therefor.

(iii) For purposes of Section 9(b)(2)(i)(i), an Award (other than Restricted Stock) shall be considered assumed if, following consummation of the Reorganization Event, such Award confers the right to purchase or receive pursuant to the terms of such Award, for each share of Common Stock subject to the Award immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); *provided*, *however*, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise or settlement of the Award to consist solely of such number of shares of common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determined to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

(3) Consequences of a Reorganization Event on Restricted Stock. Upon the occurrence of a Reorganization Event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company with respect to outstanding Restricted Stock shall inure to the benefit of the Company's successor and shall, unless the Board determines otherwise, apply to the cash, securities or other property which the Common Stock was converted into or exchanged for pursuant to such Reorganization Event in the same manner and to the same extent as they applied to such Restricted Stock; *provided*, *however*, that the Board may provide for termination or deemed satisfaction of such repurchase or other rights under the

instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, either initially or by amendment. Upon the occurrence of a Reorganization Event involving the liquidation or dissolution of the Company, except to the extent specifically provided to the contrary in the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, all restrictions and conditions on all Restricted Stock then outstanding shall automatically be deemed terminated or satisfied.

10. General Provisions Applicable to Awards.

(a) Transferability of Awards. Awards (or any interest in an Award, including, prior to exercise, any interest in shares of Common Stock issuable upon exercise of an Option or SAR) shall not be sold, assigned, transferred (including by establishing any short position, put equivalent position (as defined in Rule 16a-1 issued under the Exchange Act) or call equivalent position (as defined in Rule 16a-1 issued under the Exchange Act)), pledged, hypothecated or otherwise encumbered by the person to whom they are granted, either voluntarily or by operation of law, and, during the life of the Participant, shall be exercisable only by the Participant; except that Awards, other than Awards subject to Section 409A of the Code, may be transferred to family members (as defined in Rule 701(c)(3) under the Securities Act) through gifts or (other than Incentive Stock Options) domestic relations orders or to an executor or guardian upon the death of the Participant. The Company shall not be required to recognize any such permitted transfer until such time as such permitted transferee shall deliver to the Company a written instrument, as a condition to such transfer, in form and substance satisfactory to the Company confirming that such transferee shall be bound by all of the terms and conditions of the Award. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees. For the avoidance of doubt, nothing contained in this Section 10(a) shall be deemed to restrict a transfer to the Company.

(b) Documentation. Each Award shall be evidenced in such form (written, electronic or otherwise) as the Board shall determine. Each Award may contain terms and conditions in addition to those set forth in the Plan.

(c) Board Discretion. Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Board need not treat Participants uniformly.

(d) Termination of Status. The Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights under the Award.

(e) Withholding. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under an Award. The Company may decide to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a

broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise, vesting or release from forfeiture of an Award or at the same time as payment of the exercise or purchase price unless the Company determines otherwise. If provided for in an Award or approved by the Board in its sole discretion, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock, including shares retained from the Award creating the tax obligation, valued at their Fair Market Value; *provided*, *however*, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income). Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

(f) Amendment of Award.

(1) The Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type, changing the date of exercise or realization, and converting an Incentive Stock Option to a Nonstatutory Stock Option. The Participant's consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, does not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 9.

(2) The Board may, without stockholder approval, amend any outstanding Award granted under the Plan to provide an exercise price per share that is lower than the then-current exercise price per share of such outstanding Award. The Board may also, without stockholder approval, cancel any outstanding award (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan covering the same or a different number of shares of Common Stock and having an exercise price per share lower than the then-current exercise price per share of the cancelled award.

(g) Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to the Plan or to remove restrictions from shares previously issued or delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(h) Acceleration. The Board may at any time provide that any Award shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

11. Miscellaneous.

(a) No Right To Employment or Other Status. No person shall have any claim or right to be granted an Award by virtue of the adoption of the Plan, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Award.

(b) No Rights As Stockholder. Subject to the provisions of the applicable Award, no Participant or Designated Beneficiary shall have any rights as a stockholder with respect to any shares of Common Stock to be distributed with respect to an Award until becoming the record holder of such shares.

(c) Effective Date and Term of Plan. The Plan shall become effective on the date on which it is adopted by the Board. No Awards shall be granted under the Plan after the expiration of 10 years from the earlier of (i) the date on which the Plan was adopted by the Board or (ii) the date the Plan was approved by the Company's stockholders, but Awards previously granted may extend beyond that date.

(d) Amendment of Plan. The Board may amend, suspend or terminate the Plan or any portion thereof at any time; *provided* that if at any time the approval of the Company's stockholders is required as to any modification or amendment under Section 422 of the Code or any successor provision with respect to Incentive Stock Options, the Board may not effect such modification or amendment without such approval. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 11(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, provided the Board determines that such amendment, taking into account any related action, does not materially and adversely affect the rights of Participants under the Plan.

(e) Authorization of Sub-Plans (including Grants to non-U.S. Employees). The Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable securities, tax or other laws of various jurisdictions. The Board shall establish such sub-plans by adopting supplements to the Plan containing (i) such limitations on the Board's discretion under the Plan as the Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Board shall deem necessary or desirable. All supplements adopted by the Board shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

(f) Compliance with Section 409A of the Code. Except as provided in individual Award agreements initially or by amendment, if and to the extent (i) any portion of any payment, compensation or other benefit provided to a Participant pursuant to the Plan in connection with his or her employment termination constitutes "nonqualified deferred compensation" within the meaning of Section 409A of the Code and (ii) the Participant is a specified employee as defined

in Section 409A(a)(2)(B)(i) of the Code, in each case as determined by the Company in accordance with its procedures, by which determinations the Participant (through accepting the Award) agrees that he or she is bound, such portion of the payment, compensation or other benefit shall not be paid before the day that is six months plus one day after the date of "separation from service" (as determined under Section 409A of the Code) (the "***New Payment Date***"), except as Section 409A of the Code may then permit. The aggregate of any payments that otherwise would have been paid to the Participant during the period between the date of separation from service and the New Payment Date shall be paid to the Participant in a lump sum on such New Payment Date, and any remaining payments will be paid on their original schedule.

The Company makes no representations or warranty and shall have no liability to the Participant or any other person if any provisions of or payments, compensation or other benefits under the Plan are determined to constitute nonqualified deferred compensation subject to Section 409A of the Code but do not to satisfy the conditions of that section.

(g) Limitations on Liability. Notwithstanding any other provisions of the Plan, no individual acting as a director, officer, other employee, or agent of the Company will be liable to any Participant, former Participant, spouse, beneficiary, or any other person for any claim, loss, liability, or expense incurred in connection with the Plan, nor will such individual be personally liable with respect to the Plan because of any contract or other instrument he or she executes in his or her capacity as a director, officer, other employee, or agent of the Company. The Company will indemnify and hold harmless each director, officer, other employee, or agent of the Company to whom any duty or power relating to the administration or interpretation of the Plan has been or will be delegated, against any cost or expense (including attorneys' fees) or liability (including any sum paid in settlement of a claim with the Board's approval) arising out of any act or omission to act concerning the Plan unless arising out of such person's own fraud or bad faith.

(h) Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

PTC Therapeutics, Inc.
2013 STOCK INCENTIVE PLAN

CALIFORNIA SUPPLEMENT

Pursuant to Section 11(e) of the Plan, the Board has adopted this supplement for purposes of satisfying the requirements of Section 25102(o) of the California Law:

Any Awards granted under the Plan to a Participant who is a resident of the State of California on the date of grant (a "***California Participant***") shall be subject to the following additional limitations, terms and conditions:

1. Additional Limitations on Options.

(a) Maximum Duration of Options. No Options granted to California Participants shall have a term in excess of 10 years measured from the Option grant date.

(b) Minimum Exercise Period Following Termination. Unless a California Participant's employment is terminated for cause (as defined by applicable law, the terms of the Plan or option grant or a contract of employment), in the event of termination of employment of such Participant, such Participant shall have the right to exercise an Option, to the extent that such Participant is entitled to exercise such Option on the date employment terminated, until the earlier of: (i) at least six months from the date of termination, if termination was caused by such Participant's death or disability, (ii) at least 30 days from the date of termination, if termination was caused other than by such Participant's death or disability and (iii) the Option expiration date.

2. Additional Limitations for Other Stock-Based Awards. The terms of all Awards granted to a California Participant under Section 8 of the Plan shall comply, to the extent applicable, with Sections 260.140.42, 260.140.45 and 260.140.46 of the California Code of Regulations.

3. Additional Limitations on Timing of Awards. No Award granted to a California Participant shall become exercisable, vested or realizable, as applicable to such Award, unless the Plan has been approved by the holders of a majority of the Company's outstanding voting securities by the later of (i) within 12 months before or after the date the Plan was adopted by the Board, or (ii) prior to or within 12 months of the granting of any Award to a California Participant.

4. Additional Restriction Regarding Recapitalizations, Stock Splits, Etc. For purposes of Section 9 of the Plan, in the event of a stock split, reverse stock split, stock dividend, recapitalization, combination, reclassification or other distribution of the Company's securities underlying the Award without the receipt of consideration by the Company, the number of securities purchasable, and in the case of Options, the exercise price of such Options, must be proportionately adjusted.

5. Additional Limitations on Transferability of Awards. Notwithstanding the provisions of Section 10(a) of the Plan, an Award granted to a California Participant may not be transferred to an executor or guardian upon the disability of the Participant.

Exhibit 10.8

PTC Therapeutics, Inc.

Restricted Stock Agreement
Granted Under 2013 Stock Incentive Plan

AGREEMENT made this [ ], 2013, between PTC Therapeutics, Inc., a Delaware corporation (the "Company"), and [ ] (the "Participant").

For valuable consideration, receipt of which is acknowledged, the parties hereto agree as follows:

1. Issuance of Shares.

The Company shall issue to the Participant, and the Participant shall receive from the Company, subject to the terms and conditions set forth in this Agreement and in the Company's 2013 Stock Incentive Plan (the "Plan"), [ ] shares (the "Shares") of common stock, $0.001 par value, of the Company ("Common Stock") in exchange for past and future services provided by the Participant. The Company shall issue to the Participant one or more certificates in the name of the Participant for that number of Shares issued to the Participant. The Participant agrees that the Shares shall be subject to the forfeiture provisions set forth in Section 2 of this Agreement, the restrictions on transfer set forth in Section 3 of this Agreement and the purchase option set forth in Section 4 of this Agreement.

2. Forfeiture Provisions.

(a) Except as otherwise set forth in this Section 2, in the event that the Participant ceases to perform services for the Company for any reason or no reason, with or without cause, prior to the second anniversary of the Vesting Commencement Date (as defined below), any Unvested Shares (as defined below) as of such date shall immediately and automatically, with no action required on the part of the Company or the Participant, be forfeited back to the Company for no consideration. Upon such forfeiture, the Participant shall have no further rights with respect to the Unvested Shares.

[**For departing directors:** There shall be no Shares subject to forfeiture and therefore no "Unvested Shares".] / [**For all other recipients:** "Unvested Shares" means the total number of Shares multiplied by the Applicable Percentage at the time of the forfeiture. The "Applicable Percentage" shall be (i) 100% during the period ending on the first anniversary of the Vesting Commencement Date, (ii) 50% during the period ending on the second anniversary of the Vesting Commencement Date and (iii) zero on or after the second anniversary of the Vesting Commencement Date. For purposes of this Agreement, "Vesting Commencement Date" shall mean [ ].]

(b) Upon a Change in Control (as defined in Section 4(g)(2) below) of the Company, the vesting schedule of the Shares shall be accelerated in full so that all then Unvested

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Shares shall immediately become free from the forfeiture provisions immediately prior to such Change in Control.

(c) In the event that the Participant's service with the Company is terminated on account of the Participant's death, Disability (as defined below) or termination by the Company without Cause (as defined below), the vesting schedule of the Shares shall be accelerated in full so that all then Unvested Shares shall immediately become free from the forfeiture provisions as of the date of such termination. For purposes of this Agreement, "Disability" shall have the meaning set forth in Section 22(e)(3) of the Internal Revenue Code of 1986, as amended. For purposes of this Agreement, "Cause" shall exist upon (i) a good faith finding by the Board of Directors of the Company (A) of repeated and willful failure of the Participant after written notice to perform his or her reasonably assigned duties for the Company, or (B) that the Participant has engaged in dishonesty, gross negligence or misconduct, which dishonesty, gross negligence or misconduct has had a material adverse effect on the business or affairs of the Company; (ii) the conviction of the Participant of, or the entry of a pleading of guilty or nolo contendere by the Participant to, any crime involving moral turpitude or any felony; or (iii) a breach by the Participant of any material provision of any invention and non-disclosure agreement or non-competition and non-solicitation agreement with the Company, which breach is not cured within ten days written notice thereof.

(d) For purposes of this Agreement, service with the Company shall include service with a parent or subsidiary of the Company and service to the Company as an advisor, consultant or member of the Board of Directors of the Company.

3. Restrictions on Transfer.

(a) The Participant shall not sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively "transfer") any Shares, or any interest therein, that are subject to the forfeiture provisions set forth in Section 2 of this Agreement, except that the Participant may transfer such Shares (i) to or for the benefit of any spouse, children, parents, uncles, aunts, siblings, grandchildren and any other relatives approved by the Board of Directors (collectively, "Approved Relatives") or to a trust established solely for the benefit of the Participant and/or Approved Relatives, provided that such Shares shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in this Section 4, the forfeiture provisions set forth in Section 2 of this Agreement and the right of first refusal set forth in Section 4) and such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement or (ii) as part of the sale of all or substantially all of the shares of capital stock of the Company (including pursuant to a merger or consolidation), provided that, in accordance with the Plan, the securities or other property received by the Participant in connection with such transaction shall remain subject to this Agreement.

(b) The Participant shall not transfer any Shares, or any interest therein, that are no longer subject to the forfeiture provisions set forth in Section 2 of this Agreement, except in accordance with Section 4 below.

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4. Right of First Refusal.

(a) If the Participant proposes to transfer any Shares that are no longer subject to the forfeiture provisions set forth in Section 2 of this Agreement, then the Participant shall first give written notice of the proposed transfer (the "Transfer Notice") to the Company. The Transfer Notice shall name the proposed transferee and state the number of such Shares the Participant proposes to transfer (the "Offered Shares"), the price per share and all other material terms and conditions of the transfer.

(b) For 30 days following its receipt of such Transfer Notice, the Company shall have the option to purchase all or part of the Offered Shares at the price and upon the terms set forth in the Transfer Notice. In the event the Company elects to purchase all or part of the Offered Shares, it shall give written notice of such election to the Participant within such 30-day period. Within 10 days after his or her receipt of such notice, the Participant shall tender to the Company at its principal offices the certificate or certificates representing the Offered Shares to be purchased by the Company, duly endorsed in blank by the Participant or with duly endorsed stock powers attached thereto, all in a form suitable for transfer of the Offered Shares to the Company. Promptly following receipt of such certificate or certificates, the Company shall deliver or mail to the Participant a check in payment of the purchase price for such Offered Shares; provided that if the terms of payment set forth in the Transfer Notice were other than cash against delivery, the Company may pay for the Offered Shares on the same terms and conditions as were set forth in the Transfer Notice; and provided further that any delay in making such payment shall not invalidate the Company's exercise of its option to purchase the Offered Shares.

(c) If the Company does not elect to acquire all of the Offered Shares, the Participant may, within the 30-day period following the expiration of the option granted to the Company under subsection (b) above, transfer the Offered Shares which the Company has not elected to acquire to the proposed transferee, provided that such transfer shall not be on terms and conditions more favorable to the transferee than those contained in the Transfer Notice. Notwithstanding any of the above, all Offered Shares transferred pursuant to this Section 4 shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in Section 3 and the right of first refusal set forth in this Section 4) and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement.

(d) After the time at which the Offered Shares are required to be delivered to the Company for transfer to the Company pursuant to subsection (b) above, the Company shall not pay any dividend to the Participant on account of such Offered Shares or permit the Participant to exercise any of the privileges or rights of a stockholder with respect to such Offered Shares, but shall, insofar as permitted by law, treat the Company as the owner of such Offered Shares.

(e) The following transactions shall be exempt from the provisions of this Section 4:

(1) a transfer of Shares to or for the benefit of any Approved Relatives, or to a trust established solely for the benefit of the Participant and/or Approved Relatives;

(2) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the "Securities Act"); and

(3) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation);

provided, however, that in the case of a transfer pursuant to clause (1) above, such Shares shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in Section 3 and the right of first refusal set forth in this Section 4) and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement.

(f) The Company may assign its rights to purchase Offered Shares in any particular transaction under this Section 4 to one or more persons or entities.

(g) The provisions of this Section 4 shall terminate upon the earlier of the following events:

(1) the closing of the sale of shares of Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(2) the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company's voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 75% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction). A transaction of the type contemplated by this Section 4(g)(2) is herein referred to as a "Change in Control" of the Company.

(h) The Company shall not be required (1) to transfer on its books any of the Shares which shall have been sold or transferred in violation of any of the provisions set forth in this Agreement, or (2) to treat as owner of such Shares or to pay dividends to any transferee to whom any such Shares shall have been so sold or transferred.

5. Agreement in Connection with Initial Public Offering.

The Participant agrees, in connection with the initial underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to

purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable for shares of Common Stock or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock, whether any transaction described in clause (a) or (b) is to be settled by delivery of shares of Common Stock or other securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days from the date of the final prospectus relating to the

offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address FINRA Rule 2711(f)(4) or NYSE Rule 472(f)(4) or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the "lock-up" period.

6. Escrow.

The Participant shall, upon the execution of this Agreement, execute Joint Escrow Instructions in the form attached to this Agreement as Exhibit A. The Joint Escrow Instructions shall be delivered to the Secretary of the Company, as escrow agent thereunder. The Participant shall deliver to such escrow agent a stock assignment duly endorsed in blank, in the form attached to this Agreement as Exhibit B, and hereby instructs the Company to deliver to such escrow agent, on behalf of the Participant, the certificate(s) evidencing the Shares issued hereunder. Such materials shall be held by such escrow agent pursuant to the terms of such Joint Escrow Instructions.

7. Restrictive Legends.

All certificates representing Shares shall have affixed thereto legends in substantially the following form, in addition to any other legends that may be required under federal or state securities laws:

> "The shares of stock represented by this certificate are subject to forfeiture provisions, restrictions on transfer and an option to purchase set forth in a certain Restricted Stock Agreement between the corporation and the registered owner of these shares (or his predecessor in interest), and such Agreement is available for inspection without charge at the office of the Secretary of the corporation."
>
> "The shares represented by this certificate have not been registered under the Securities Act of 1933, as amended, and may not be sold, transferred or otherwise disposed of in the absence of an effective registration statement under such Act or an opinion of counsel

satisfactory to the corporation to the effect that such registration is not required."

8. Provisions of the Plan.

This Agreement is subject to the provisions of the Plan, a copy of which is furnished to the Participant with this Agreement.

9. Investment Representations.

The Participant represents, warrants and covenants as follows:

(a) The Participant is purchasing the Shares for his own account for investment only, and not with a view to, or for sale in connection with, any distribution of the Shares in violation of the Securities Act, or any rule or regulation under the Securities Act.

(b) The Participant has had such opportunity as he has deemed adequate to obtain from representatives of the Company such information as is necessary to permit him to evaluate the merits and risks of his investment in the Company.

(c) The Participant has sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Shares and to make an informed investment decision with respect to such purchase.

(d) The Participant can afford a complete loss of the value of the Shares and is able to bear the economic risk of holding such Shares for an indefinite period.

(e) The Participant understands that (i) the Shares have not been registered under the Securities Act and are "restricted securities" within the meaning of Rule 144 under the Securities Act; (ii) the Shares cannot be sold, transferred or otherwise disposed of unless they are subsequently registered under the Securities Act or an exemption from registration is then available; (iii) in any event, the exemption from registration under Rule 144 will not be available for at least one year and even then will not be available unless a public market then exists for the Common Stock, adequate information concerning the Company is then available to the public, and other terms and conditions of Rule 144 are complied with; and (iv) there is now no registration statement on file with the Securities and Exchange Commission with respect to any stock of the Company and the Company has no obligation or current intention to register the Shares under the Securities Act.

10. Withholding Taxes; Section 83(b) Election.

(a) The Participant acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Participant any federal, state or local taxes of any kind required by law to be withheld with respect to the issuance of the Shares by the Participant or the lapse of the forfeiture provisions set forth in Section 2 of this Agreement.

(b) The Participant has reviewed with the Participant's own tax advisors the federal, state, local and foreign tax consequences of this investment and the transactions contemplated by this Agreement. The Participant is relying solely on such advisors and not on any statements or representations of the Company or any of its agents. The Participant understands that the Participant (and not the Company) shall be responsible for the Participant's own tax liability that may arise as a result of this investment or the transactions contemplated by this Agreement. The Participant understands that it may be beneficial in many circumstances to elect to be taxed at the time the Shares are granted by the Company rather than when and as the forfeiture provisions set forth in Section 2 of this Agreement expire by filing an election under Section 83(b) of the Internal Revenue Code of 1986 with the I.R.S. within 30 days from the date of grant by the Company.

THE PARTICIPANT ACKNOWLEDGES THAT IT IS SOLELY THE PARTICIPANT'S RESPONSIBILITY AND NOT THE COMPANY'S TO FILE TIMELY THE ELECTION UNDER SECTION 83(b), EVEN IF THE PARTICIPANT REQUESTS THE COMPANY OR ITS

REPRESENTATIVES TO MAKE THIS FILING ON THE PARTICIPANT'S BEHALF.

11. Miscellaneous.

(a) No Rights to Service. The Participant acknowledges and agrees that the vesting of the Shares pursuant to Section 2 hereof is earned only by continuing service as an employee at the will of the Company (not through the act of being hired or purchasing shares hereunder), advisor to the Company or member of the Company's Board of Directors. The Participant further acknowledges and agrees that the transactions contemplated hereunder and the vesting schedule set forth herein do not constitute an express or implied promise of continued engagement as an employee, director or advisor for the vesting period, for any period, or at all.

(b) Severability. The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement, and each other provision of this Agreement shall be severable and enforceable to the extent permitted by law.

(c) Waiver. Any provision for the benefit of the Company contained in this Agreement may be waived, either generally or in any particular instance, by the Board of Directors of the Company.

(d) Binding Effect. This Agreement shall be binding upon and inure to the benefit of the Company and the Participant and their respective heirs, executors, administrators, legal representatives, successors and assigns, subject to the restrictions on transfer set forth in Sections 3 and 4 of this Agreement.

(e) Notice. All notices required or permitted hereunder shall be in writing and deemed effectively given upon personal delivery or five days after deposit in the United States Post Office, by registered or certified mail, postage prepaid, addressed to the other party hereto at the address shown beneath his or its respective signature to this Agreement, or at such other

address or addresses as either party shall designate to the other in accordance with this Section 11(e).

(f) Pronouns. Whenever the context may require, any pronouns used in this Agreement shall include the corresponding masculine, feminine or neuter forms, and the singular form of nouns and pronouns shall include the plural, and vice versa.

(g) Entire Agreement. This Agreement and the Plan constitute the entire agreement between the parties, and supersedes all prior agreements and understandings, relating to the subject matter of this Agreement.

(h) Amendment. This Agreement may be amended or modified only by a written instrument executed by both the Company and the Participant.

(i) Governing Law. This Agreement shall be construed, interpreted and enforced in accordance with the internal laws of the State of Delaware without regard to any applicable conflict of law principles.

(j) Participant's Acknowledgments. The Participant acknowledges that he or she: (i) has read this Agreement; (ii) has been represented in the preparation, negotiation, and execution of this Agreement by legal counsel of the Participant's own choice or has voluntarily declined to seek such counsel; (iii) understands the terms and consequences of this Agreement; (iv) is fully aware of the legal and binding effect of this Agreement; and (v) understands that the law firm of WilmerHale is acting as counsel to the Company in connection with the transactions contemplated by the Agreement, and is not acting as counsel for the Participant.

[Remainder of Page Intentionally Left Blank]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

By: _____
Name: _____
Title: _____
Address: _____

PARTICIPANT:

_____
Name:
Address: _____

Exhibit A

Joint Escrow Instructions

PTC Therapeutics, Inc.

Joint Escrow Instructions

[, 20]

PTC Therapeutics, Inc.
Middlesex Business Center
100 Corporate Court
South Plainfield, NJ 07080

Attn: Secretary
Dear Sir:

As Escrow Agent for PTC Therapeutics, Inc., a Delaware corporation (the "Company"), and its successors in interest under the Restricted Stock Agreement (the "Agreement") of even date herewith, to which a copy of these Joint Escrow Instructions is attached, and the undersigned person ("Holder"), you are hereby authorized and directed to hold the documents delivered to you pursuant to the terms of the Agreement in accordance with the following instructions:

1. Appointment. Holder irrevocably authorizes the Company to deposit with you any certificates evidencing Shares (as defined in the Agreement) to be held by you hereunder and any additions and substitutions to said Shares. For purposes of these Joint Escrow Instructions, "Shares" shall be deemed to include any additional or substitute property. Holder does hereby irrevocably constitute and appoint you as his attorney-in-fact and agent for the term of this escrow to execute with respect to such Shares all documents necessary or appropriate to make such Shares negotiable and to complete any transaction herein contemplated. Subject to the provisions of this Section 1 and the terms of the Agreement, Holder shall exercise all rights and privileges of a stockholder of the Company while the Shares are held by you.

2. Closing of Purchase.

(a) Upon any purchase by the Company of the Shares pursuant to the Agreement, the Company shall give to Holder and you a written notice specifying the number of Shares to be purchased, the purchase price for the Shares, as determined pursuant to the Agreement, and the time for a closing hereunder (the "Closing") at the principal office of the Company. Holder and the Company hereby irrevocably authorize and direct you to close the transaction contemplated by such notice in accordance with the terms of said notice.

(b) At the Closing, you are directed (i) to date the stock assignment form or forms necessary for the transfer of the Shares, (ii) to fill in on such form or forms the number of Shares being transferred, and (iii) to deliver the same, together with the certificate or certificates

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evidencing the Shares to be transferred, to the Company against the simultaneous delivery to you of the purchase price for the Shares being purchased pursuant to the Agreement.

3. Withdrawal. The Holder shall have the right to withdraw from this escrow any Shares as to which forfeiture provisions set forth in Section 2 of the Agreement have terminated or expired.

4. Duties of Escrow Agent.

(a) Your duties hereunder may be altered, amended, modified or revoked only by a writing signed by all of the parties hereto.

(b) You shall be obligated only for the performance of such duties as are specifically set forth herein and may rely and shall be protected in relying or refraining from acting on any instrument reasonably believed by you to be genuine and to have been signed or presented by the proper party or parties. You shall not be personally liable for any act you may do or omit to do hereunder as Escrow Agent or as attorney-in-fact of Holder while acting in good faith and in the exercise of your own good judgment, and any act done or omitted by you pursuant to the advice of your own attorneys shall be conclusive evidence of such good faith.

(c) You are hereby expressly authorized to disregard any and all warnings given by any of the parties hereto or by any other person or entity, excepting only orders or process of courts of law, and are hereby expressly authorized to comply with and obey orders, judgments or decrees of any court. If you are uncertain of any actions to be taken or instructions to be followed, you may refuse to act in the absence of an order, judgment or decrees of a court. In case you obey or comply with any such order, judgment or decree of any court, you shall not be liable to any of the parties hereto or to any other person or entity, by reason of such compliance, notwithstanding any such order, judgment or decree being subsequently reversed, modified, annulled, set aside, vacated or found to have been entered without jurisdiction.

(d) You shall not be liable in any respect on account of the identity, authority or rights of the parties executing or delivering or purporting to execute or deliver the Agreement or any documents or papers deposited or called for hereunder.

(e) You shall be entitled to employ such legal counsel and other experts as you may deem necessary properly to advise you in connection with your obligations hereunder and may rely upon the advice of such counsel.

(f) Your rights and responsibilities as Escrow Agent hereunder shall terminate if (i) you cease to be Secretary of the Company or (ii) you resign by written notice to each party. In the event of a termination under clause (i), your successor as Secretary shall become Escrow Agent hereunder; in the event of a termination under clause (ii), the Company shall appoint a successor Escrow Agent hereunder.

(g) If you reasonably require other or further instruments in connection with these Joint Escrow Instructions or obligations in respect hereto, the necessary parties hereto shall join in furnishing such instruments.

(h) It is understood and agreed that if you believe a dispute has arisen with respect to the delivery and/or ownership or right of possession of the securities held by you hereunder, you are authorized and directed to retain in your possession without liability to anyone all or any part of said securities until such dispute shall have been settled either by mutual written agreement of the parties concerned or by a final order, decree or judgment of a court of competent jurisdiction after the time for appeal has expired and no appeal has been perfected, but you shall be under no duty whatsoever to institute or defend any such proceedings.

(i) These Joint Escrow Instructions set forth your sole duties with respect to any and all matters pertinent hereto and no implied duties or obligations shall be read into these Joint Escrow Instructions against you.

(j) The Company shall indemnify you and hold you harmless against any and all damages, losses, liabilities, costs, and expenses, including attorneys' fees and disbursements, (including without limitation the fees of counsel retained pursuant to Section 4(e) above, for anything done or omitted to be done by you as Escrow Agent in connection with this Agreement or the performance of your duties hereunder, except such as shall result from your gross negligence or willful misconduct.

5. Notice. Any notice required or permitted hereunder shall be given in writing and shall be deemed effectively given upon personal delivery or upon deposit in the United States Post Office, by registered or certified mail with postage and fees prepaid, addressed to each of the other parties thereunto entitled at the following addresses, or at such other addresses as a party may designate by ten days' advance written notice to each of the other parties hereto.

COMPANY: Notices to the Company shall be sent to the address set forth in the salutation hereto, Attn: President

HOLDER: Notices to Holder shall be sent to the address set forth below Holder's signature below.

ESCROW AGENT: Notices to the Escrow Agent shall be sent to the address set forth in the salutation hereto.

6. Miscellaneous.

(a) By signing these Joint Escrow Instructions, you become a party hereto only for the purpose of said Joint Escrow Instructions, and you do not become a party to the Agreement.

(b) This instrument shall be binding upon and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

Very truly yours,

PTC Therapeutics, Inc.

By: _____
Name: _____
Title: _____

HOLDER:

_____
Name:

Address: _____

_____

Date Signed: _____

ESCROW AGENT:

_____
Secretary

Exhibit B

Stock Assignment Separate from Certificate

(Stock Assignment Separate from Certificate)

FOR VALUE RECEIVED, I hereby sell, assign and transfer unto ( ) shares of Common Stock, $0.001 par value per share, of PTC Therapeutics, Inc. (the "Corporation") standing in my name on the books of the Corporation represented by Certificate(s) Number herewith, and do hereby irrevocably constitute and appoint Wilmer Cutler Pickering Hale and Dorr LLP attorney to transfer the said stock on the books of the Corporation with full power of substitution in the premises.

Dated: _____

_____

Name:

NOTICE: The signature(s) to this assignment must correspond with the name as written upon the face of the certificate, in every particular, without alteration, enlargement, or any change whatever.

Exhibit 10.9

PTC Therapeutics, Inc.

Nonstatutory Stock Option Agreement
Granted Under 2013 Stock Incentive Plan

1. Grant of Option.

This agreement evidences the grant by PTC Therapeutics, Inc., a Delaware corporation (the "Company"), on [ ], 2013 (the "Grant Date") to **[ ]**, an employee, consultant or director of the Company (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's 2013 Stock Incentive Plan (the "Plan"), a total of [ ] shares (the "Shares") of common stock, $[ ] par value per share, of the Company ("Common Stock") at $[ ] per Share. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on March 4, 2023 (the "Final Exercise Date").

It is intended that the option evidenced by this agreement shall not be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule; Exercise Period.

This option shall be fully vested on the Grant Date.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the Final Exercise Date.

3. Exercise of Option.

Each election to exercise this option shall be accompanied by a completed Notice of Stock Option Exercise in the form attached hereto as Exhibit A, signed by the Participant, and received by the Company at its principal office, accompanied by this agreement, and payment in full in the manner provided in the Plan. The Participant may purchase less than the number of Shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

4. Company Right of First Refusal.

(a) Notice of Proposed Transfer. If the Participant proposes to sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively, "transfer") any Shares acquired upon exercise of this option, then the Participant shall first give written notice of the proposed transfer (the "Transfer Notice") to the Company. The Transfer Notice shall name the proposed transferee and state the number of such Shares the Participant

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proposes to transfer (the "Offered Shares"), the price per share and all other material terms and conditions of the transfer.

(b) Company Right to Purchase. For 30 days following its receipt of such Transfer Notice, the Company shall have the option to purchase all or part of the Offered Shares at the price and upon the terms set forth in the Transfer Notice. In the event the Company elects to purchase all or part of the Offered Shares, it shall give written notice of such election to the Participant within such 30-day period. Within 10 days after his or her receipt of such notice, the Participant shall tender to the Company at its principal offices the certificate or certificates representing the Offered Shares to be purchased by the Company, duly endorsed in blank by the Participant or with duly endorsed stock powers attached thereto, all in a form suitable for transfer of the Offered Shares to the Company. Promptly following receipt of such certificate or certificates, the Company shall deliver or mail to the Participant a check in payment of the purchase price for such Offered Shares; provided that if the terms of payment set forth in the Transfer Notice were other than cash against delivery, the Company may pay for the Offered Shares on the same terms and conditions as were set forth in the Transfer Notice; and provided further that any delay in making such payment shall not invalidate the Company's exercise of its option to purchase the Offered Shares.

(c) Shares Not Purchased By Company. If the Company does not elect to acquire all of the Offered Shares, the Participant may, within the 30-day period following the expiration of the option granted to the Company under subsection (b) above, transfer the Offered Shares which the Company has not elected to acquire to the proposed transferee, provided that such transfer shall not be on terms and conditions more favorable to the transferee than those contained in the Transfer Notice. Notwithstanding any of the above, all Offered Shares transferred pursuant to this Section 4 shall remain subject to the right of first refusal set forth in this Section 4 and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Section 4.

(d) Consequences of Non-Delivery. After the time at which the Offered Shares are required to be delivered to the Company for transfer to the Company pursuant to subsection (b) above, the Company shall not pay any dividend to the Participant on account of such Offered Shares or permit the Participant to exercise any of the privileges or rights of a stockholder with respect to such Offered Shares, but shall, insofar as permitted by law, treat the Company as the owner of such Offered Shares.

(e) Exempt Transactions. The following transactions shall be exempt from the provisions of this Section 4:

(1) any transfer of Shares to or for the benefit of any spouse, child or grandchild of the Participant, or to a trust for their benefit;

(2) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the "Securities Act"); and

(3) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation);

provided, however, that in the case of a transfer pursuant to clause (1) above, such Shares shall remain subject to the right of first refusal set forth in this Section 4.

(f) Assignment of Company Right. The Company may assign its rights to purchase Offered Shares in any particular transaction under this Section 4 to one or more persons or entities.

(g) Termination. The provisions of this Section 4 shall terminate upon the earlier of the following events:

(1) the closing of the sale of shares of Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(2) the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company's voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 75% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction).

(h) No Obligation to Recognize Invalid Transfer. The Company shall not be required (1) to transfer on its books any of the Shares which shall have been sold or transferred in violation of any of the provisions set forth in this Section 4, or (2) to treat as owner of such Shares or to pay dividends to any transferee to whom any such Shares shall have been so sold or transferred.

(i) Legends. The certificate representing Shares shall bear a legend substantially in the following form (in addition to, or in combination with, any legend required by applicable federal and state securities laws and agreements relating to the transfer of the Company securities):

> "The shares represented by this certificate are subject to a right of first refusal in favor of the Company, as provided in a certain stock option agreement with the Company."

5. Agreement in Connection with Initial Public Offering.

The Participant agrees, in connection with the initial underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in

whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address FINRA Rule 2711(f)(4) or NYSE Rule 472(f)(4) or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the "lock-up" period.

6. Withholding.

No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option.

7. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4 and Section 5; provided that such a written confirmation shall not be required with respect to (1) Section 4 after such provision has terminated in accordance with Section 4(g) or (2) Section 5 after the completion of the lock-up period in connection with the Company's initial underwritten public offering.

8. Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

IN WITNESS WHEREOF, the Company has caused this option to be executed under its corporate seal by its duly authorized officer. This option shall take effect as a sealed instrument.

PTC Therapeutics, Inc.

By: _____

Name:
Title:

Signature Page to Nonstatutory Stock Option Agreement

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's 2013 Stock Incentive Plan.

PARTICIPANT:

_____

Address: _____

_____

SPOUSAL CONSENT:

_____

Name: _____

Address: _____

_____

NOTICE OF STOCK OPTION EXERCISE

Date: [ ](2)

PTC Therapeutics, Inc.
Middlesex Business Center
100 Corporate Court
South Plainfield, NJ 07080

Attention: Treasurer

Dear Sir or Madam:

I am the holder of a Nonstatutory Stock Option granted to me under the PTC Therapeutics, Inc. (the "Company") 2013 Stock Incentive Plan on March 5, 2013 for the purchase of [ ](3) shares of Common Stock of the Company at a purchase price of $[ ](4) per share.

I hereby exercise my option to purchase [ ](5) shares of Common Stock (the "Shares"), for which I have enclosed [ ](6) in the amount of [ ](7). Please register my stock certificate as follows:

[Name(s): _____(8)

_____

Address: _____

_____]

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(2) Enter date of exercise.
(3) Enter the total number of shares of Common Stock for which the option was granted.
(4) Enter the option exercise price per share of Common Stock.
(5) Enter the number of shares of Common Stock to be purchased upon exercise of all or part of the option.
(6) Enter "cash", "personal check" or if permitted by the option or Plan, "stock certificates No. XXXX and XXXX".
(7) Enter the dollar amount (price per share of Common Stock times the number of shares of Common Stock to be purchased), or the number of shares tendered. Fair market value of shares tendered, together with cash or check, must cover the purchase price of the shares issued upon exercise.
(8) Enter name(s) to appear on stock certificate: (a) Your name only; (b) Your name and other name (i.e., John Doe and Jane Doe, Joint Tenants With Right of Survivorship); or (c) In the case of a Nonstatutory option only, a Child's name, with you as custodian (i.e., Jane Doe, Custodian for Tommy Doe). Note: There may be income and/or gift tax consequences of registering shares in a Child's name.

I represent, warrant and covenant as follows:

1. I am purchasing the Shares for my own account for investment only, and not with a view to, or for sale in connection with, any distribution of the Shares in violation of the Securities Act of 1933 (the "Securities Act"), or any rule or regulation under the Securities Act.

2. I have had such opportunity as I have deemed adequate to obtain from representatives of the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company.

3. I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Shares and to make an informed investment decision with respect to such purchase.

4. I can afford a complete loss of the value of the Shares and am able to bear the economic risk of holding such Shares for an indefinite period.

5. I understand that (i) the Shares have not been registered under the Securities Act and are "restricted securities" within the meaning of Rule 144 under the Securities Act, (ii) the Shares cannot be sold, transferred or otherwise disposed of unless they are subsequently registered under the Securities Act or an exemption from registration is then available; (iii) in any event, the exemption from registration under Rule 144 will not be available for at least one year and even then will not be available unless a public market then exists for the Common Stock, adequate information concerning the Company is then available to the public, and other terms and conditions of Rule 144 are complied with; and (iv) there is now no registration statement on file with the Securities and Exchange Commission with respect to any stock of the Company and the Company has no obligation or current intention to register the Shares under the Securities Act.

Very truly yours,

_____

(Signature)

Exhibit 10.13

TABLE OF CONTENTS
OF LEASE BETWEEN 46.24 ASSOCIATES L.P., AS LANDLORD,
AND PTC THERAPEUTICS, INC., AS TENANT, FOR PREMISES LOCATED IN 100
CORPORATE COURT, MIDDLESEX BUSINESS CENTER, SOUTH PLAINFIELD, NEW
JERSEY

[ILLEGIBLE]	LL
[ILLEGIBLE]	T

THIS LEASE, made as of the 1th day of July 2000, by and between 46.24 ASSOCIATES L.P., a Delaware limited partnership, having a mailing address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord") and PTC THERAPEUTICS, INC., a Massachusetts corporation, having its principal office at 2 Chestnut Street, Grafton, Massachusetts 01519 (hereinafter referred to as "Tenant").

WITNESSETH:

WHEREAS, the Landlord has constructed a building (hereinafter referred to as "Building") for the purposes of office use, known as Building No. 100 located within the area designated as Lot No. 46.24 (hereinafter referred to as "Lot No. 46.24") on the attached plot plan (hereinafter referred to as "Plot Plan") which is annexed hereto as Exhibit "A" and made a part hereof; and

WHEREAS, Landlord and the owner of the area designated as Lot Not 46.25 (hereinafter referred to as "Lot No. 46.25") on the Plot Plan each has the right to operate and use the Common Areas (as hereinafter defined) within Lots 46.24 and 46.25; and

WHEREAS, Tenant is desirous of leasing from Landlord and Landlord is desirous of leasing to Tenant certain premises in the Building which is situated within MIDDLESEX BUSINESS CENTER (hereafter referred to as "Center") hereinafter described, upon and subject to the provisions, agreements, covenants and conditions set forth herein;

NOW, THEREFORE, it is mutually agreed as follows:

ARTICLE 1. DEMISED PREMISES AND TERM

Section 1.01.(a) In consideration of the rents and additional rents hereinafter reserved and all of the provisions, agreements, covenants and conditions hereinafter contained, Landlord hereby leases and demises to Tenant, and Tenant hereby hires, leases and takes from Landlord approximately 21,700 square feet of floor space ("Floor Space") in the Building, more particularly indicated and described by cross-hatching on the Plot Plan (such Floor Space being hereinafter referred to as the "Demised Premises") located on Lot No. 46.24 in the Center located in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX and STATE OF NEW JERSEY, together with all improvements to be constructed thereon by the Landlord for the use of the Tenant, and all easements, tenements and appurtenances thereto.

Section 1.01.(b) The parties acknowledge that the Landlord intends to erect or has erected other buildings on Lot No. 46.24 (which may be different in design and construction from the Building) which buildings may be constructed at the sole option of Landlord. Landlord shall have sole control and discretion in connection with the scope, design and aesthetics of any such additional construction. Notwithstanding the foregoing, any such other buildings and improvements associated therewith shall not unreasonably interfere with access to the Demised Premises or the loading area thereof, nor reduce the number of parking spaces below the number required by municipal code.

Section 1.01.(c) The Demised Premises are demised and let subject to (i) the existing state of the title thereof; (ii) any state of facts which an accurate survey or physical inspection thereof might disclose; (iii) all zoning regulations, restrictions, rules and ordinances now in effect or hereafter adopted by any governmental authority having jurisdiction; and (iv) any utility, sewer or drainage easements or agreements and the installations made pursuant thereto now existing or hereafter granted or installed; all without representation or warranty by Landlord, except as expressly set forth herein. Notwithstanding the foregoing, Landlord hereby represents, that, to the best of Landlord's knowledge: (a) the Building is presently zoned to permit the use of the Demised Premises for typical office/laboratory purposes; and (b) any existing utility, sewer or drainage easements or agreements and the installations made pursuant thereto will not unreasonably interfere with Tenant's use and occupancy of the Demised Premises. Landlord further represents to Tenant that Landlord

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is presently the fee owner of the Building and is authorized and empowered to enter into this Lease as Landlord and to perform the obligations of Landlord hereunder.

Section 1.02. As long as Tenant occupies the Demised Premises, Tenant, together with its employees, customers, invitees and business guests, shall have the right to use, in common with Landlord, its successors, assigns, tenants, subtenants, designees, concessionaires, licensees and any of their customers, invitees, and business guests, all of the Common Areas (as such term is defined in Section 12.01 hereof) at any time and from time to time existing within Lot No. 46.24, except for areas reserved for the exclusive use of other tenants, occupants, or designees and except for periods of time during which the Common Areas are being repaired, altered or reconstructed. Neither Landlord nor Tenant nor anyone holding under or through either of them shall make any charge for the use of the Common Areas to the other or to the employees, customers, invitees or business guests of Landlord or Tenant or of anyone else hereinbefore granted the right to use the Common Areas, except as provided in Article 12 of this Lease.

Section 1.03. The term ("Term") of this Lease shall commence on that date ("the Commencement Date") which is the first to occur of: (a) the date upon which the Demised Premises are first occupied by Tenant for the conduct of business operations at the Demised Premises, or (b) the date which is thirty (30) days following the date upon which the Landlord's Work (as hereinafter defined) shall be duly certified by Landlord or Landlord's agent as being substantially completed, except for those items the completion of which will not unreasonably interfere with Tenant's use and occupancy of the Demised Premises as provided herein, and shall expire on the date which is the FIVE (5) years following the last day of the calendar month in which the Rent Commencement Date (as hereinafter defined) shall occur ("Expiration Date"). Landlord represents to Tenant that Landlord's Work shall be performed in accordance with all applicable codes so as to enable Tenant to apply for and obtain a certificate of occupancy for the Demised Premises' upon completion of Tenant's improvements and Tenant's fixturing and equipping of the Demised Premises. Tenant may, at any time after execution of this Lease and prior to the Commencement Date, without incurring any liability for payment of annual minimum rental or additional rent, measure the Demised Premises, design and layout of the tenant improvements and Tenant's Property (hereinafter defined), and place and install its personal property, furniture, furnishings, signs, telecommunication equipment, equipment and trade fixtures ("Tenant's Property"), in the Demised Premises at Tenant's risk and expense. In exercising the foregoing rights, Tenant shall not cause any material interference with or delay to Landlord, and Tenant's indemnity provided for in this Lease shall apply to Tenant's entry under this Section.

Section 1.04. The parties shall, within ten (10) days following request of the other, execute a written document, in recordable form, expressing the Commencement Date and Expiration Date of the Term hereof, as such have been determined in accordance with the provisions of this Lease.

Section 1.05. The term "Lease Year" is defined to mean twelve (12) consecutive calendar months; the first Lease Year to commence on the first day of the succeeding calendar month following the Commencement Date and each succeeding Lease Year to commence on the anniversary date of the commencement of the first Lease Year. The portion of the Term prior to the first Lease Year shall be deemed a "Partial Lease Year" and any obligations of Tenant for such Partial Lease Year shall be prorated on a per diem basis.

ARTICLE 2. USE AND OPERATION

Section 2.01. Subject to the other provisions of this Lease, Tenant shall occupy and use the Demised Premises solely for office and laboratory purposes and incidental uses normally associated therewith, and for no other use. Tenant hereby covenants and agrees that it, its successors and assigns, or anyone holding by, through or under them, shall not use, nor permit the use of the Demised Premises for any other use or purpose. Immediately following certification under Section 1.03 above, Tenant shall fixture, furnish and equip the Demised Premises for Tenant's intended business purpose and upon the Commencement Date, Tenant shall occupy and open for business in the Demised Premises.

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T [ILLEGIBLE]

ARTICLE 3. RENT

Section 3.01. The annual minimum rental during the Term shall be as follows:

(A) During the FIRST (1st) and SECOND (2nd) years following the Rent Commencement Date; ONE HUNDRED EIGHTY FOUR THOUSAND FOUR HUNDRED FIFTY AND 00/100 ($184,450.00) DOLLARS per annum — FIFTEEN THOUSAND THREE HUNDRED SEVENTY AND 00/100 ($15,370.00) DOLLARS per month;

(B) During the THIRD (3rd) year following the Rent Commencement Date: ONE HUNDRED NINETY FIVE THOUSAND THREE HUNDRED AND 00/100 ($195,300.00) DOLLARS per annum — SIXTEEN THOUSAND TWO HUNDRED SEVENTY FIVE AND 00/100 ($16,275.00) DOLLARS per month; and

(C) During the balance of the Term of this Lease; TWO HUNDRED SIX THOUSAND ONE HUNDRED FIFTY AND 00/100 ($206,150.00) per annum — SEVENTEEN THOUSAND ONE HUNDRED SEVENTY NINE AND 17/100 ($17,179.17) DOLLARS per month.

The "Rent Commencement Data" shall be the [ILLEGIBLE] day occurring following the expiration of the "Rent Concession Period", in accordance with the provisions hereinafter act forth in Section 5.05 of the this Lease, or as otherwise determined in accordance with said Section 5.05.

Tenant

By /s/ SWP

Landlord

By /s/ [ILLEGIBLE]

All annual minimum rental payable under this Lease during the Term hereof shall be paid to Landlord in advance, on the first day of each calendar month during the Term hereof at the office of Landlord or such other place or to such other person or party as Landlord may designate, without prior demand therefor and without any setoff or deduction whatsoever, except as heroin provided. Annual minimum rent and additional rent shall be prorated for a fraction of a month, if any, based on the number of days within such fractional month. Unless and until otherwise designated by Landlord in writing all annual minimum rent and additional rent payable under this Lease shall be paid to National Realty & Development Corp., at 3 Manhattanville Road, Purchase, New York 10577.

Section 3.02. All taxes, charges, costs and expenses which Tenant assumes or agrees to pay under any provision of this Lease, together with any and all other gums and legal fees which may become due, by reason of any default of Tenant or failure on Tenant's part to comply with the provisions, covenants and conditions of this Lease on Tenant's part to be performed, and each or any of them, shall be collectible and recoverable as additional rent, and, in the event of nonpayment thereof, Landlord shall have all the rights and remedies herein provided as in the case of nonpayment of annual minimum rent.

ARTICLE 4. SUBORDINATION

Section 4.01. This Lease and all right of Tenant hereunder are, and shall be, subject and subordinate to any mortgages, deeds of trust (including blanket mortgages or deeds of trust covering the Demised Premises and/or the Center and/or other properties) or any other security interest which lies been or which hereinafter may affect the Demised Premises, and to any ground or underlying leases of all or part of the Canter, and to any renewals, modifications, consolidations, replacements and extensions thereof (hereinafter collectively referred to as "Landlord's Financing"). Landlord represents that, as of the date hereof, the sole holder of Landlord' s Financing is The Travelers Life and Annuity Company, One Tower square, 2 SHS, Hartford, Connecticut 06183. Tenant acknowledges that the interest of Landlord under this Lease may be assigned by Landlord as collateral security to any of the foregoing partier holding interest to which this Lease is subject and subordinate. In the event of foreclosure of any such interest, or termination of any such ground or underlying lease, or in the event of an exercise of the power of sale under any mortgage or other security interest made by Landlord covering the promises of which the Demised Premises forms a part, Tenant shall, at the sale option and direction of any such party, recognize the rights of any such party under and pursuant to the provisions of such collateral assignment and/or allom to and

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T [ILLEGIBLE]

recognize any purchaser at a foreclosure sale of any mortgage or deed of trust or any such purchaser at a sale exercised in connection with the mortgagee's or trustee's remedy of power of sale pursuant to any mortgage or deed of trust affecting the Demised Premises and/or Center or any transferee who acquires the Demised Premises and/or Center by deed in lieu of foreclosure, and the successors and/or assigns of such transferee or purchaser. Notwithstanding anything to the contrary contained herein, this Lease shall not be subject and subordinate to the lien of Landlord's Financing, unless an instrument, duly executed by the holder(s) of Landlord's Financing, shall be delivered to Tenant which instrument shall contain an agreement in substance, to be effective only so long as Tenant shall not be in default under the provisions of this Lease, that such holder shall recognize Tenant's rights under this Lease, shall not cut off or terminate this Lease through foreclosure of the documents securing Landlord's Financing, and Tenant shall not be disturbed in its possession of the Demised Premises or the exercise of any of its rights under the Lease, which agreement may also contains such provisions as are typically included therein by commercial lenders (such agreement hereinafter referred to as the "Subordination, Non-Disturbance and Attornment Agreement"). The Subordination, Non-Disturbance and Attornment Agreement shall be in recordable form and in substance reasonably satisfactory to Tenant, Landlord and the holder(s) of Landlord's Financing. Tenant acknowledges that the form annexed hereto as Exhibit D is satisfactory to Tenant.

Section 4.02. The provisions of Section 4.01 shall be self-operative, but Tenant covenants and agrees that it shall, within ten (10) days following request, at any time or times, execute, acknowledge and deliver to Landlord any instruments in order to subordinate this Lease and Tenant's rights hereunder, as aforesaid, said instruments to be in the form required by any mortgagee, ground lessor or other secured party.

Section 4.03. If Tenant shall fail or neglect to execute, acknowledge and deliver any documents required by this Article, Landlord, in addition to any other remedies, may, as agent or attorney-in-fact of Tenant, execute, acknowledge and deliver same on behalf of Tenant, and Tenant hereby irrevocably nominates, constitutes and appoints Landlord as Tenant's proper and legal attorney-in-fact for such purpose, hereby ratifying all such acts that Landlord may do as attorney-in-fact of Tenant.

Section 4.04. Tenant shall, at any time and from time to time, upon not less than ten (10) days prior notice, execute, acknowledge and deliver to Landlord a statement in writing certifying that this Lease is unmodified and in full force and effect (or, if there have been modifications, that the same is in full force and effect, as modified, and stating the modifications) and the dates to which the rent and other charges have been paid in advance, if any, and stating whether or not Landlord is in default in the performance of any provision, covenant or condition contained in this Lease, and if so, specifying each such default, and containing

any other statements or certifications required by a mortgagee, and/or ground lessor and/or other secured party, it being intended that any statement or certification delivered pursuant to this Section may be relied upon by any party to whom it may be delivered by Landlord.

Section 4.05. The ground and underlying leases and mortgages referred to in this Article 4 to which this Lease is subject and subordinate are hereinafter sometimes called "superior leases" and "superior mortgages", respectively, and the lessor of a superior lease, or its successor in interest at the time, is hereinafter sometimes called the "lessor" of such superior lease. No pre-payment of more than one month's rent shall be valid or binding upon the holder of a superior mortgage or the lessor of a superior lease unless expressly approved in writing by such holder or lessor or by any of its predecessors in interest.

Section 4.06. Tenant agrees not to look to the mortgagee, as mortgagee, mortgagee in possession, or successor in title to the Demised Premises and/or the Center, for accountability for any security deposit required by Landlord hereunder, unless said sums have actually been received by said mortgagee as security for Tenant's performance of this Lease.

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ARTICLE 5. AS-IS; LANDLORD'S WORK;TENANT'S WORK

Section 5.01. Tenant has examined the Demised Premises and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the Demised Premises, except as is in this Lease specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to the best of its knowledge and except as disclosed in the Phase I Environmental Assessment prepared by Environmental Management Services dated June 19, 2000, there are no environmental hazards present within the Demised Premises. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation, examination, and inspection of the Demised Premises and Tenant agrees to accept the Demised Premises "as is", except that Tenant shall replace non-functioning horns, strobes and pullboxes in the Demised Premises, provided, however, Landlord shall reimburse Tenant for the one-half of the cost of replacing non-functioning horns, strobes and pullboxes in the Demised Premises (Landlord reimbursement not to exceed $3,000), subject to Landlord's receipt and approval of the estimates for such work, which approval shall not be unreasonably withheld or delayed, and receipt by Landlord of satisfactory documentation evidencing the payment and lien-free completion of such work.

Section 5.02. Landlord or Landlord's contractor may give Tenant notice that the Landlord's Work is substantially complete to the extent that it is practicable for Tenant to enter therein for the performance of work by Tenant necessary to occupy the Demised Premises and open for business, and if such notice shall be given, Tenant shall promptly thereafter commence all work that is necessary to open the Demised Premises for business. Subject to the foregoing provisions of this Section, Tenant shall have the right to install its fixtures and equipment during construction, provided Tenant does not interfere with the construction of the Demised Premises or Building, and, further, provided, that insurance meeting the requirements of Section 7.02 is furnished to Landlord prior to any such entry. Such entry into the Demised Premises by Tenant prior to the Commencement Date is and shall be at the Tenant's sole cost and risk, and the provisions of Section 7.01 and Section 7.02 shall be applicable during any such period prior to the Commencement Date. Prior to commencing any work, Tenant shall give Landlord prior written notice of the date on which Tenant intends to commence such work, which notice shall describe the type of labor (i.e. union or non-union labor) Tenant intends to hire for its work. Notwithstanding anything herein to the contrary, Landlord may require Tenant to use union labor for all work performed in the Demised Premises, the Building and/or in the Center if, in Landlord's reasonable judgment, the use of non-union labor would delay or interfere with the progress of any construction within the Building and/or Center and/or the operation of any business in the Building and/or Center. In the event that Landlord does not initially require Tenant to use union labor and a labor dispute subsequently arises due to Tenant's use of non-union labor, then Tenant shall, within twenty-four (24) hours following notice from Landlord (which may be oral or written), cause each conflicting labor to leave the Demised Premises, the Building and Center, and thereafter Tenant shall prosecute its work only with local union labor. All fixturing and/or other work to be performed by or on behalf of Tenant (other than Landlord's work hereunder) shall be done in accordance with plans and specifications therefor submitted to and approved by Landlord prior to the commencement of such fixturing and/or other work, which approval shall not be unreasonably withheld, and in accordance with and subject to the provisions of Article 19 hereof. No changes shall be made in said plans and specifications nor shall there be any deviation in the prosecution of the work in accordance with said plans and specifications without Landlord's prior written approval.

Section 5.03. If Tenant claims that some or all of the construction requirements imposed upon Landlord pursuant to the provisions of this Lease have not been complied with by Landlord upon delivery of notice of substantial completion of Landlord's Work, as provided herein, Tenant shall, within ten (10) days of said date (or ten days following the date Tenant opens for the transaction of business, whichever date is sooner), submit to Landlord a written list of the work Tenant claims remains to be performed by Landlord, and Landlord shall have ninety (90) days thereafter to complete such work. If Landlord fails to complete such work, the sole remedy of

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Tenant shall be to complete such work and Tenant shall have the right to set off the cost thereof from the rent due Landlord in order to reimburse Tenant for the cost and expense of completion of the work. Upon written request of Landlord, Tenant will, within five (5) days following request (but not sooner than the date required by the first sentence of this Section), furnish to Landlord a written statement that Tenant is in occupancy of the Demised Premises, that the construction of the Demised Premises has been completed in accordance with Landlord's obligations or in lieu thereof, a list of the work Tenant claims to be incomplete. Notwithstanding the foregoing, the aforesaid time period shall not be applicable to latent defects in the Landlord's Work for which notice may be given to Landlord promptly following the date upon which Tenant discovers, or reasonably could have discovered, any such latent defects. Landlord agrees to assign to Tenant all warranties relating to the Landlord's Work, provided, however, the Landlord reserves the right to enforce same, jointly with, or independently of Tenant.

Section 5.04. Landlord shall substantially complete the construction of the Landlord's Work on or prior to that date which is one hundred twenty (120) days following the date of issuance of a building permit for the Landlord's Work to be performed at the Demised Premises.

If possession of the substantially completed Demised Premises shall not be delivered to Tenant on or prior to such date, Tenant shall have the right to cancel this Lease upon notice to Landlord to be given within fifteen (15) days following such date, unless the substantially completed Demised Premises shall be delivered to Tenant prior thereto. If Tenant shall not exercise such right of cancellation, the date by which Landlord is obligated to deliver possession of the

Demised Premises shall be deemed to be automatically extended for an additional period of ninety (90) days. If possession of the substantially completed Demised Premises is not delivered to Tenant prior to the expiration of such extension period, or if Tenant shall cancel this Lease pursuant to the first sentence of this paragraph, this Lease shall automatically terminate and be null and void and of no further force and effect, and the parties shall be mutually released of and from all rights and obligations hereunder. Tenant's right to cancel this Lease, as provided herein, shall be Tenant's sole remedy for Landlord's failure to deliver possession on or before the required date.

If the substantial completion of the Landlord's Work is delayed by reason of: (i) any act or omission of Tenant or any of its employees, agents or contractors; or (ii) any failure (not due to any act or omission of Landlord or any of its employees, agents or contractors) to plan or execute Tenant's work necessary for Tenant's occupancy of the Demised Premises with reasonable speed and diligence, or (iii) any changes by Tenant in the plans or specifications for the construction of the Demised Premises or any changes or substitutions requested by Tenant; or (iv) Tenant's failure to furnish plans and specifications required to be furnished by Tenant, or subsequent changes thereto; or (v) Tenant's request for materials, finishes or installations other than Landlord's typical building standard; or (vi) the performance or incompletion of work by a party employed or retained by Tenant; then the Landlord's Work shall be deemed substantially completed on the date when the same would have been substantially completed but for such delay and, in addition, Tenant shall pay to Landlord all costs and damages which Landlord may sustain by reason of such delay.

Section 5.05. (A) Tenant, at Tenant's sole cost and expense, subject to the Tenant Allowance (as hereinafter defined), shall perform all work necessary to renovate, fixture and equip the Demised Premises for Tenant's use in accordance with the provisions of Section 2.01 of this Lease (the "Tenant Improvements"). Tenant covenants and agrees that Tenant shall commence the performance of the Tenant Improvements within fifteen (15) days following the issuance of a building permit, and thereafter Tenant shall diligently prosecute such Tenant Improvements and complete same as soon as possible, but in no event more than three (3) months from and after the date that the building permit shall have been issued ("Tenant Improvement Completion Date"). Tenant agrees to apply for the building permit immediately following Tenant's receipt of notice from Landlord that Landlord has approved the plans and specifications for the performance of the Tenant Improvements.

Section 5.05 (B) Subject to completion of Tenant Improvements in accordance with Tenant's Plans (as hereinafter defined) and the provisions set forth below in this Section 5.05(B), Landlord shall reimburse Tenant for amounts actually expended by Tenant for a portion of the construction of the Tenant Improvements, in an amount not to exceed the sum of $217,000.00 (the "Tenant

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Allowance"). Such reimbursement shall be in the form of a credit to be taken by Tenant against the first payments of annual minimum rental payable hereunder. The portion of the Tenant Improvements for which the Tenant Allowance shall be paid shall be only that portion thereof that is office installation work (e.g. partitioning, doors, electrical, etc., including, without limitation, upgrading and replacing of existing mechanical systems in the Demised Premises) and in no event shall the Tenant Allowance be used for any work related to Tenant's trade fixtures or equipment (such portion of the Tenant Improvements for which Tenant may receive the Tenant Allowance is hereinafter called the "Tenant Allowance Work"). Supplementing the foregoing, Landlord acknowledges and agrees that the work described in Exhibit E annexed hereto shall be deemed to be Tenant Allowance Work. Pending the completion of the Tenant Allowance Work (which "completion" shall be deemed to include the delivery to Landlord of the documentation required pursuant to Subparagraph (C) of this Section 5.05, the annual minimum rental payable pursuant to Section 3.01 of this Lease shall be abated (the period for which annual minimum rental is abated is referred to herein as the "Rent Concession Period"). Notwithstanding anything to the contrary set forth herein, in the event that the Tenant Improvements have not been completed and the documentation required pursuant to Subparagraph (C) of this Section 5.05 have not been delivered to Landlord on or before the Tenant Improvement Completion Date, Tenant shall make immediate payment to Landlord of any monthly minimum rental payments that have been abated during the Rent Concession Period, the Rent Commencement Date shall be deemed to be the Commencement Date of this Lease and Tenant will immediately commence payment of annual minimum rental amounts as set forth in 3.01. Landlord shall be entitled to draw upon the letter of credit for the purpose of collecting such unpaid minimum rental payments. Notwithstanding the foregoing, in the event that the completion of the Tenant Improvements and the delivery to Landlord of the documentation required pursuant to Subparagraph (C) of this Section 5.05 has not occurred by the Tenant Improvement Completion Date by reason of the causes set forth in Section 5.06 below, then the Tenant Improvement Completion Date shall be extended for such period of delay, but in no event shall any such delay extend for more than an additional three (3) months following the Tenant Improvement Completion Date. It is further acknowledged and agreed by Tenant that in the event that the monies expended by Tenant for the completion of the Tenant Allowance Work are less than the amount of the Tenant Allowance, Tenant shall not be entitled to any credit for the unused portion thereof, nor is Tenant entitled to any additional sums from Landlord in the event that the monies expended by Tenant in connection with the completion of the Tenant Allowance Work or the Tenant Improvements exceeds the amount of the Tenant Allowance.

Section 5.05. (C) The Tenant Improvements shall be effected solely in accordance with the plans and specifications approved by Landlord (such plans and specifications hereinafter referred to as "Tenant's Plans"). The Tenant Improvements shall be performed in accordance with the foregoing and the following provisions of this Article 5:

1. All work shall be done in a good and workmanlike manner.

2. Tenant and its contractor and any subcontractor shall agree to employ only such labor as will not result in jurisdictional disputes or strikes or result in causing disharmony with other workers employed at the Building. Tenant will inform Landlord in writing of the names of any contractor or subcontractor Tenant proposes to use in the Demised Premises within a reasonable time prior to the beginning of work by such contractor or subcontractor. A copy of any contract or subcontract affecting Tenant Improvements, the Demised Premises or the Building shall be submitted to Landlord prior to commencement of the work provided for therein.

3. The Tenant Improvements shall be effected in compliance with all applicable laws, ordinances, rules and regulations of governmental bodies having or asserting jurisdiction thereover and Tenant shall procure and furnish to Landlord copies of all governmental permits and authorizations which may be required in connection with such work, prior to the commencement thereof.

4. Tenant, at its expense, and with diligence and dispatch, shall procure the cancellation or discharge of all notices of violation arising from or otherwise connected with work performed or alleged to have been performed by its contractor(s) or subcontractor(s) and which shall be issued by

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the Borough of South Plainfield or any other public authority having or asserting jurisdiction. Tenant shall defend, indemnify and save harmless Landlord against any and all mechanic's and other liens and financing or title retention devices filed in connection therewith and against all costs, expenses and liabilities (including reasonable attorney's fees) incurred in connection with any such lien, financing statement, conditional bill of sale, chattel mortgage or other financing or title retention devices, or any action or proceeding brought thereon. Tenant shall keep the Land, the Building and the Demised Premises free and clear of all liens for any work or material claimed to have been furnished to Tenant or to the Demised Premises on Tenant's behalf, and all work to be performed by Tenant shall be done in a manner which will not interfere with or disturb other tenants or occupants of the Building. All notices of intention, mechanic's liens, financing statements, conditional bills of sale, chattel mortgages and other financing or title retention devices filed against Tenant, the Demised Premises, or the Building for work claimed to have been done for or materials claimed to have been furnished to Tenant shall be cancelled and discharged of record by Tenant at its expense within ten (10) days after such filing, by payment or filing of the bond required by law.

Neither Tenant nor any of its agents, employees, representatives, contractors or subcontractors shall have any power or authority to do any act or thing or to make any contract or agreement which will bind Landlord or which may create or be the foundation for any mechanic's lien or other lien or claim upon or against Landlord or Landlord's interest in the Real Property; and further, Landlord shall have no responsibility to Tenant or to any architect, engineer, contractor, subcontractor, supplier, material man, workman or other person, firm or corporation who shall engage in or participate in the performance of Tenant Improvements, any additional work or any installation, alteration or improvement to be performed or made by Tenant under any of the terms of this Lease, or otherwise, unless Landlord shall expressly undertake such obligation by an agreement in writing, signed by Landlord, and made between Tenant and Landlord or such other party. Notice is hereby given that Landlord shall not be liable for any labor or materials furnished or to be furnished to Tenant upon credit and that no mechanic's or other lien for any such labor or materials shall attach to or affect the reversion or other estate or interest of Landlord in and to the Demised Premises, the Land and/or the Building. A copy of the foregoing provisions of this paragraph shall be included in any contract entered into by or on behalf of Tenant for the performance of or the furnishing or installation of Tenant Work, any additional work, or any installation, alteration or improvement to the Demised Premises.

5. During the progress of the work to be done by Tenant, said work shall be subject to inspection by representatives of Landlord, which shall be permitted access and the opportunity to inspect at all reasonable times, but this provision shall not in any way whatsoever create any obligation on Landlord to conduct an inspection or impose any liability on Landlord for the failure of any such work.

6. Prior to commencement of any Tenant Work, Tenant or Tenant's contractor shall furnish to Landlord policies of insurance or certificates thereof evidencing the existence of the insurance coverages required by Section 7.02 hereof, including without limitation comprehensive general liability and builder's risk insurance. Upon request by Landlord, Tenant's contractors and/or subcontractors shall furnish to Landlord Performance Bonds and/or Completion Bonds, in form and substance reasonably satisfactory to Landlord.

7. Upon completion of the work, Tenant shall, at its sole cost and expense, remove all debris from the Demised Premises and the Building, and clean the same. If it is necessary for Landlord to do any work to clean the Building and/or the Center or any part thereof, Tenant shall reimburse Landlord, upon demand therefor, Landlord's cost of cleaning same, plus Landlord supervision charges of fifteen (15%) percent.

8. Upon completion of Tenant Improvements, Tenant shall submit to Landlord in form and substance satisfactory to Landlord and counsel for Landlord the following:

(a) A Certificate of Completion by a licensed architect or engineer, which Certificate shall certify that all Tenant Improvements has been completed in accordance with the approved plans and specifications;

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(b) A certificate by Tenant, or if Tenant is a corporation by an executive officer of Tenant, that the entire cost of Tenant Improvements has been paid and the amount thereof, that all those who furnished work or materials have been paid in full, and that no party has filed any lien or possesses any claim which is unpaid or remains undischarged;

(c) A Certificate of Occupancy, or an equivalent permit or certificate, required by any governmental authorities prior to opening for business within the Demised Premises;

(d) A final release and lien waiver signed by Tenant's general contractor and all subcontractors and materialmen, together with a certificate by the general contractor to the effect that all those who furnished work or materials to the Demised Premises have been paid in full and that the release and waiver has been signed by all those who furnished work or materials to the Demised Premises; and

(e) Final and complete "as-built" plans (architectural and mechanical) for the Demised Premises.

Section 5.06. If there shall be a delay in the completion of the Landlord's Work or repair or restoration of the Demised Premises or Center or any portion thereof caused by strikes, riots, acts of God, shortages of labor or materials, national emergency, governmental restrictions, laws or regulations, the act or failure to act of Tenant, including without limitation, delays in delivering construction criteria and plan approval, or for any other cause or causes beyond Landlord's control, at Landlord's option such delay shall not be a violation of this Lease, and the time periods set forth in this Lease for any such work shall, at Landlord's option, be extended for a period of time equal to the period of delay.

Section 5.07. The Plot Plan shows the approximate location of existing buildings, buildings under construction, proposed buildings and certain areas reserved for related site improvements and future construction at the option of Landlord. Landlord shall have the right to develop the Center in the manner it sees fit and in the sole and absolute discretion of Landlord: to construct or not construct any buildings other than the Building, to change the nature or identity of the occupants of any such buildings, and to vary the floor areas, stories and heights, sizes, shapes and design of any such buildings and the divisions or portions thereof.

ARTICLE 6. ALTERATIONS AND REPAIRS

Section 6.01. No alterations or additions shall at any time be made by or at the instance of Tenant without Landlord's prior written consent, except that Tenant may make alterations, the cost or value of which is not greater than $10,000.00, without Landlord's consent, provided such alterations do not affect the

exterior or load bearing structural integrity of the Building or materially or adversely affect the building systems serving the Demised Premises. All work, repairs, and/or alterations made by or at the instance of Tenant shall be done in a good and workmanlike manner, with first class materials, in compliance with any applicable governmental rules and regulations, and subject to Article 19 hereof, and the cost thereof shall be paid by Tenant in cash or its equivalent, so that the Demised Premises shall at all times be free of liens for labor or materials supplied or claimed to have been supplied to the Demised Premises. Any alterations, installations, repairs, additions or improvements (inclusive of paneling and other wall coverings), except Tenant's trade fixtures, shall, at the option of Landlord, become the property of Landlord and shall remain upon and be surrendered with the Demised Premises, as part thereof, at the expiration or sooner termination of the Term. If Tenant is in default hereunder or is dispossessed, or vacates the premises, voluntarily or otherwise, and fails to remove any property, equipment and fixtures within ten (10) days following notice by Landlord, then and in that event, the said property, equipment and fixtures shall be deemed, at the option of Landlord, to be abandoned; or in lieu thereof, at the Landlord's option, Landlord may remove and store or dispose of such property and charge the cost and expense of removal, storage and disposal to Tenant. , provided, however, Landlord's option to elect that Such alterations either remain with the Demised Premises or be removed at the Tenant's expense, and the Landlord's determination as to whether any particular fixtures are "trade fixtures", shall be made

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within thirty days (30) following Tenant's written request to Landlord to make such determination with respect to any particular installation of any of such alterations or fixtures. Trade fixtures shall be defined as fixtures and equipment used by Tenant in the operation of its business, but not including any fixtures and equipment which are part of the operation of the Demised Premises or the Building.

Section 6.02. Anything to the contrary contained herein notwithstanding, it is expressly understood and agreed that Tenant may install, connect and operate such machinery, fixtures and equipment as may be deemed necessary by the Tenant for its business, subject to compliance with applicable rules and regulations of governmental bodies and bureaus having jurisdiction thereover. Subject to the terms and conditions of this Lease, the machinery, fixtures and equipment belonging to Tenant shall, at all times, be considered and intended to be personal property of Tenant, and not part of the realty, and subject to removal by Tenant, provided, at the time of such removal, that Tenant is not in default pursuant to any of the terms, covenants, provisions or conditions of this Lease. Tenant, at its own cost and expense, shall pay for any damage to the Demised Premises or Building caused by the installation thereof or such removal, and this obligation shall survive the expiration or sooner termination of the Term.

Section 6.03. Landlord shall, following reasonable notice from Tenant, make all necessary repairs and replacements to the exterior structural portions of the Demised Premises, including the roof and foundations thereof, provided, however, Landlord shall not be required to make any repairs or replacements caused by any act, omission, or negligence of Tenant, any subtenant, or concessionaire, or their respective employees, agents, invitees, licensees or contractors, including any repairs to the roof necessitated by roof penetrations made by Tenant, it being understood that Landlord shall be responsible for any roof repairs arising out of Landlord's Work. Tenant shall make all other repairs and replacements to the Demised Premises. Tenant shall maintain throughout the Term, including any extension term hereof, a protective service maintenance contract with a contractor approved by Landlord, which approval shall not be unreasonably withheld, providing for periodic maintenance of the H.V.A.C. system serving the Demised Premises, including without limitation periodic changing of any and all filters, changing of belts, lubricating of equipment and maintenance of operating levels of freon in accordance with manufacturers specifications. Said contract shall provide for maintenance inspection and service not less than two (2) times per year. A copy of any such maintenance contract shall be delivered to Landlord on a yearly basis or more often if required by Landlord. Tenant shall keep all glass clean and in good condition, and Tenant shall replace any glass which may be damaged or broken with glass of the same quality. Tenant shall keep the sidewalk, if any, adjacent to the Demised Premises free and clear of trash, litter and rubbish.

Section 6.04. To the extent permitted by law, nothing contained in this Lease shall authorize Tenant to do any act which may create or be the foundation for any lien, mortgage or other encumbrance upon the reversion or other estate of Landlord, or of any interest of Landlord in the Demised Premises, or upon or in the Building or Center of which the same form a part; it being agreed that should Tenant cause any alterations, changes, additions, installations, improvements or repairs to be made to the Demised Premises, or cause materials to be furnished or labor to be performed therein or thereon, neither Landlord nor the Demised Premises shall, under any circumstances, be liable for the payment of any expense incurred or for the value of any work done or materials furnished to the Demised Premises or any part thereof. Tenant shall, upon request of Landlord, deliver such documents as may be required by Landlord in order to effectuate the lien protection required by this paragraph and Section 6.01 hereof, including without limitation, waivers of lien in advance from all contractors. All such alterations, changes, additions, improvements, repairs, materials and labor shall be at Tenant's sole expense and Tenant shall be solely and wholly responsible to contractors, subcontractors, laborers and materialmen furnishing labor and material to the Demised Premises and Building or any part thereof. If, because of any act or omission of Tenant, any mechanic's or other lien or order for the payment of money shall be filed against the Demised Premises or the Building or improvements thereon or therein, or upon the Center, or against Landlord (whether or not such lien or order is valid or enforceable as such), Tenant shall, at Tenant's own cost and expense, within ten (10) days after notice of the filing thereof, cause the same to be canceled and discharged of record, or furnish Landlord with a surety bond issued by a surety

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company reasonably satisfactory to Landlord, protecting Landlord from any loss because of nonpayment of such lien or claim, and Tenant hereby indemnifies and saves harmless Landlord from and against any and all costs, expenses, claims, losses or damages, including reasonable counsel fees, resulting therefrom or by reason thereof.

Section 6.05. Except for the repair obligations of Landlord under Section 6.03 above and the restoration obligations of Landlord under and as set forth in Articles 8 and 10 hereof, the Tenant shall take good care of the Demised Premises and, at its cost and expense, keep and maintain in good repair the interior and exterior of the Demised Premises, including, but not limited to the air conditioning and heating plant, the plumbing pipes and fixtures belonging thereto; and shall repair or replace all mechanical and working parts used in connection with the air conditioning, electrical, heating and plumbing plants, fixtures and systems; and shall keep the water and sewer pipes and connections, including the gutters, leaders, and roof drains, free from other obstructions; and shall generally maintain and repair the interior and exterior of the Demised Premises and shall, at the end or the expiration of the Term (or Extension Term, whichever is applicable), deliver up the Demised Premises in good order and condition, damages by the elements, ordinary wear and tear excepted. Tenant covenants and agrees that it shall not cause or permit any waste (other than reasonable wear and tear), damage or disfigurement to the Demised Premises, or any overloading of the floors of the Building.

Section 6.06. Landlord hereby acknowledges that Tenant may obtain financing for its laboratory and/or office equipment, and that in such event, Tenant may grant a security interest to such lender or equipment lessor in connection with such financing and/or leasing. Landlord further acknowledges that such financing or leasing by Tenant shall not be deemed to be a violation of the provisions of Section 6.04 so long as the lien of such lender or equipment lessor extends solely to the trade fixtures or other property of the Tenant located at the Demised Premises, and does not attach to, affect in any manner whatsoever, the Building or property of the Landlord located at the Building, including, without limitation, the building fixtures and equipment located in the Demised Premises.

ARTICLE 7. INDEMNITY AND INSURANCE

Section 7.01. (a) To the extent not covered by the insurance required to be maintained by Landlord hereunder, Tenant hereby indemnifies and saves harmless Landlord from and against any claims and all loss, cost, liability, damage and/or expense, including, but not limited to reasonable counsel fees, penalties and fines, incurred in connection with or arising from (i) any default by Tenant in the observance or performance of any of the provisions, covenants or conditions of this Lease on Tenant's part to be observed or performed, (ii) the use or occupancy or manner of use or occupancy of the Demised Premises by Tenant or any person claiming through or under Tenant, or (iii) any acts, omissions, or negligence of Tenant or any such person, or any contractor, agent, servant, employee, visitor or licensee of Tenant, or any such person, in or about the Demised Premises. If any action or proceeding shall be brought against Landlord based upon any such claim, Tenant, upon notice from Landlord, shall cause such action or proceeding to be defended, at Tenant's expense, by counsel acting for Tenant's insurance carriers in connection with such defense or by other counsel reasonably satisfactory to Landlord.

Section 7.01. (b) To the extent not covered by insurance required to be maintained by Tenant hereunder, Landlord hereby indemnifies and saves harmless Tenant from and against any claims and all loss, cost, liability, damage and/or expense, including, but not limited to reasonable counsel fees, penalties and fines, incurred in connection with or arising from (i) any default by Landlord in the observance or performance of any of the provisions, covenants or conditions of this Lease on Landlord's part to be observed or performed, or (ii) any acts, omissions, or negligence of Landlord or any such person, or any contractor, agent, servant, employee, visitor or licensee of Landlord, or any such person, in or about the Demised Premises. If any action or proceeding shall be brought against Tenant based upon any such claim, Landlord, upon notice from Tenant, shall cause such action or proceeding to be defended, at Landlord's expense, by counsel acting for Landlord's insurance carriers in connection with such defense or by other counsel reasonably satisfactory to Tenant.

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Section 7.02. Tenant shall, during the Term (including any extension term) and during any period prior to the commencement of the Term during which Tenant or anyone acting by or on behalf of Tenant enters the Demised Premises, at Tenant's own cost and expense, maintain and provide: (a) comprehensive general liability insurance for the benefit and protection of Landlord and Tenant (said policy to name Landlord, ground lessor, if any, and any other parties designated by Landlord, as co-insureds) in an amount not less than $1,000,000 for injuries or death to any one person, and not less than $3,000,000 for injuries or death to more than one person in any one accident or occurrence and for damage to property in an amount not less than $500,000 arising out of any one accident or occurrence; (b) plate glass insurance covering all plate glass in the Demised Premises; (c) worker's compensation insurance covering all persons employed in connection with Tenant's use and occupancy of the Demised Premises or any construction or alteration work therein; (d) insurance against loss or damage to Tenant's contents, including without limitation, trade fixtures and equipment, by fire, lightning, and other risks from time to time included under standard "extended coverage" policies, and vandalism and malicious mischief, in amounts sufficient to prevent Landlord and Tenant from becoming co-insurers of any loss under such policy, but in any event, not less than 100 percent of the full insurable value of such property; (e) boiler and pressure vessel insurance on all of Tenant's equipment, parts thereof and appurtenances attached or connected to the Demised Premises which by reason of their use or existence are capable of bursting, erupting, collapsing or exploding, in the minimum amount of Five Hundred Thousand ($500,000.00) Dollars for damage to property resulting from such perils; and (f) insurance covering such other risks as may be reasonably requested by Landlord occasioned by or attributable to the use or occupancy or manner of use or occupancy of the Demised Premises by Tenant. Said policies shall be issued by companies satisfactory to Landlord and licensed to do business in the state in which the Demised Premises is located. Said policies or certificates thereof shall be delivered to Landlord at the commencement of the Term (or prior thereto in the event of earlier entry by Tenant upon the Demised Premises), together with proof of payment of premium therefor, and renewal policies or certificates therefor shall be delivered to Landlord not less than twenty (20) days prior to the expiration dates thereof. Said policies and/or certificates shall contain an undertaking by the insurer to give Landlord not less than twenty (20) days written notice of any cancellation or change in scope or amount of coverage of said policies.

Section 7.03. (a) Landlord shall, during the Term, maintain and provide general hazard insurance during the course of construction (including "builder's risk endorsements") against loss or damage to the Building by fire, lightning and other risks from time to time included under standard "Extended Coverage" policies, vandalism and malicious mischief, in amounts not less than 100 percent of the full replacement value of the Building and any other building or portion thereof covered by such insurance and rent loss insurance covering all minimum and additional rental payable hereunder. Tenant shall pay its proportionate share of the cost of maintaining and providing such insurance, which proportionate share shall be a fraction having as its numerator the number of square feet of floor area within the Demised Premises and as its denominator, the total number of square feet of floor area of all buildings within Lot 46.24.

Section 7.03. (b) Such payment shall be made to Landlord, at Landlord's Option, either annually within thirty (30) days of demand therefor or in monthly installments on or before the first day of each calendar month, in advance, in an amount estimated by Landlord. Periodically, Landlord shall furnish Tenant with a written statement of the actual amount of Tenant's proportionate share of said insurance costs. If the total amount paid by Tenant under this section for any period during the Lease Term shall be less than the actual amount due from Tenant for such period, as shown on such statement, Tenant shall pay to Landlord the difference between the amount paid by Tenant and the actual amount due, such deficiency to be paid within ten (10) clays after demand therefor by Landlord; and if the total amount paid by Tenant hereunder for any such period shall exceed the actual amount due from Tenant for such period, the excess shall promptly be applied by Landlord to the next accruing monthly installments thereof or, at Landlord's option, to any other charges payable by Tenant. For the calendar years in which this Lease commences and terminates, the provisions of this section shall apply and Tenant's liability for its proportionate share thereof for such years shall be subject to a pro rata adjustment based on the number of days of said calendar years during the Lease Term. Prior to or at the commencement of the Lease Term and from time to time

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thereafter throughout the Lease Term, Landlord will notify Tenant in writing of Landlord's estimate of Tenant's monthly installments due hereunder. Tenant's obligations under this section shall survive the expiration of the Lease Term.

Section 7.04. Insurance coverages required of Tenant hereunder shall be reviewed on an annual basis and Landlord may require that said coverages shall be updated in accordance with the provisions hereinabove set forth as to amounts and scope of coverage.

Section 7.05. In the event of any insured loss covered under the terms and conditions of this Lease, and for which Tenant is obligated to maintain insurance coverage, all insurance carriers' checks in satisfaction of the same shall be made payable to the Landlord and the holder of the first mortgage covering the Building and Tenant waives any and all rights to be designated a payee on such loss payment, except as to losses under the coverage in Section 7.02(d) above.

ARTICLE 8. FIRE DAMAGE

Section 8.01. If the Demised Premises shall be partially damaged by fire or other insured casualty, the damages shall be repaired by and at the expense of Landlord and the annual minimum rental until such repairs shall be made shall abate equitably according to the part of the Demised Premises which is unusable by Tenant or, if by reason thereof, the Demised Premises are rendered untenantable, said rental shall totally abate until such repairs shall be made. Notwithstanding the foregoing, if the Demised Premises or the Building shall be damaged to such extent that Landlord shall decide to demolish same, or not to rebuild same, then, and in such event, Landlord may terminate this Lease upon notice to Tenant given within ninety (90) days following such event, and upon the date specified in such notice, which date shall not be less than thirty (30) days nor more than sixty (60) days following the giving of said notice, this Lease shall terminate and Tenant shall vacate and surrender the Demised Premises to Landlord. Any annual minimum rental prepaid by Tenant beyond said date shall be promptly refunded to Tenant. Notwithstanding any of the foregoing provisions of this Article, if Landlord or the holder of any superior mortgage shall be unable to collect all of the insurance proceeds (including rent insurance proceeds) applicable to damage or destruction of the Demised Premises or the Building by fire or other cause, by reason of some action or inaction on the part of the Tenant or any of its employees, agents or contractors, then, without prejudice to any other remedies which may be available against Tenant, the abatement of Tenant's rents provided for in this Article shall not be effective to the extent of the uncollected insurance proceeds.

Section 8.02. If this Lease shall not be terminated as provided above in this Article, Landlord shall, at its expense, proceed with the restoration of the Demised Premises, provided, Landlord's obligations hereunder shall not exceed the scope of Landlord's initial construction obligations under this Lease and further provided, that Landlord's restoration obligations shall be subject to building and zoning laws then in effect. No penalty shall accrue for reasonable delay which may arise by reason of adjustment of insurance on the part of Landlord. Landlord shall use diligent efforts to adjust insurance promptly. If Landlord shall so restore the Demised Premises, Tenant shall repair, restore and redecorate the Demised Premises and reoccupy and reopen the Demised Premises, within fifteen (15) days following notice of restoration, in a manner and to the condition existing prior to the event of damage, except to the extent that Landlord is obligated above, and Tenant shall hold in trust the proceeds of all insurance carried by Tenant on its property for the purpose of such repair and restoration.

Section 8.03. Nothing hereinabove contained with respect to Tenant's right to abate the rent under proper conditions shall be construed to limit or affect Landlord's right to payment under the rental loss coverage to be provided pursuant to Section 7.03 hereof.

ARTICLE 9. WAIVER OF SUBROGATION

Section 9.01. Landlord, its officers, agents, employees, subsidiaries and affiliated entities

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and corporations shall not be liable for any damage to or destruction of any of Tenant's goods, merchandise, fixtures, furniture or property of whatsoever nature, caused by fire or any other cause whatsoever, including, without limitation, the negligence of any such parties, and Tenant hereby releases and waives any right of recovery against Landlord, its officers, agents, employees, subsidiaries and affiliated entities and corporations for any such loss. Tenant shall procure a waiver of subrogation on the part of the insurer against such parties by an endorsement to all insurance policies whereby the insurer recognizes the provisions of this Article.

Section 9.02. Tenant, its officers, agents, employees, subsidiaries and affiliated entities and corporations shall not be liable for any damage to or destruction of any of Landlord's goods, merchandise, fixtures, furniture or property of whatsoever nature, caused by fire or any other cause whatsoever, including, without limitation, the negligence of any such parties, and Landlord hereby releases and waives any right of recovery against Tenant, its officers, agents, employees, subsidiaries and affiliated entities and corporations for any such loss. Landlord shall procure a waiver of subrogation on the part of the insurer against such parties by an endorsement to all insurance policies whereby the insurer recognizes the provisions of this Article.

ARTICLE 10. CONDEMNATION

Section 10.01. If the whole of the Demised Premises shall be taken by any governmental authority under the power of condemnation, eminent domain, or expropriation, or in the event of a conveyance in lieu thereof, the Term shall cease as of the day possession shall be taken by such governmental authority. If more than 25 percent of the Demised Premises shall be so taken or conveyed, either Landlord or Tenant shall have the right to terminate this Lease upon notice to the other party, effective as of the day possession shall be taken by such governmental authority. If this Lease is so terminated, annual minimum rental shall be prorated as of the date that possession must be surrendered to the condemning authority.

Section 10.02. If this Lease continues after a partial taking, the annual minimum rental shall abate equitably as to the part of the Demised Premises which is taken. If this Lease continues after any such taking or conveyance, Landlord shall make all necessary repairs and restorations so as to restore the remainder of the Demised Premises to a complete architectural unit. Landlord's reconstruction obligations shall not exceed the amount of the award or compensation for the taking, shall not exceed the scope of Landlord's initial construction obligations hereunder, and shall be subject to building and zoning laws then in effect.

Section 10.03. If so much of the Center, Common Areas or Building shall be so taken or conveyed so that in the reasonable exercise of Landlord's judgment, the continued operation of the Building for use by its tenants is unfeasible, then, in such event, Landlord may, by notice to Tenant, delivered not later than thirty (30) days following the date that possession of the premises taken or conveyed is delivered to the governmental authority, terminate this Lease, and rent shall be pro rated as of the date that possession must be surrendered to the condemning authority.

Section 10.04. Tenant and not Landlord shall be entitled to any portion of the award made to Tenant for the value of Tenant's removable trade fixtures and equipment other than equipment necessary for the operation of the Building. All compensation awarded for the taking of the Building, the fee and the leasehold shall belong to and be the property of Landlord, and Tenant shall not be entitled to and hereby waives any damages for the unexpired portion of the Term, or injury to its leasehold interest.

ARTICLE 11. ASSIGNMENT AND SUBLETTING

Section 11.01. Tenant, for itself, its heirs, distributees, executors, administrators, legal representatives, successors and assigns, as the case may be, expressly covenants that it shall not assign, mortgage or encumber this agreement, nor sublet or underlet nor suffer or permit the Demised Premises or any part thereof to be used by others without the prior written consent of Landlord in each instance. If, with consent of Landlord, this Lease may be assigned, or the Demised Premises or any part thereof be underlet or occupied by anybody other than Tenant, Landlord may collect rent from the assignee, undertenant or occupant and apply the amount collected to the rent herein reserved, but no such assignment, underletting, occupancy or collecting shall be deemed to relieve Tenant or any guarantor of this Lease or guarantor of the obligations of Tenant hereunder of any of its or their obligations hereunder nor be deemed a waiver of this covenant, or the acceptance of the assignee, undertenant or occupant as tenant; or a release of Tenant or any guarantor of this Lease or any guarantor of the obligations of Tenant hereunder from its or their obligations under the covenants, provisions and conditions hereof; it being understood and agreed that Tenant and any guarantor of this Lease or any guarantor of the obligations of Tenant hereunder shall at all times, including during any extension term, remain obligated as primary obligors under this Lease. The consent by Landlord to an assignment or underletting shall not in any wise be construed to relieve Tenant or any other Tenant, assignee, undertenant, or occupant of the Demised Premises from obtaining the express consent in writing of Landlord to any further assignment or underletting, and no such assignment or subletting shall be made to anyone who shall occupy the Demised Premises for any use other than as specifically permitted by the terms of this Lease. Notwithstanding anything contained in this Lease to the contrary, in the event that it shall be found by a court of competent jurisdiction that Landlord was unreasonable in withholding its consent to the assignment of this Lease or the subletting of all or any portion of the Demised Premises, Tenant's sole remedy shall be limited to specific performance and Tenant shall not be entitled to damages or any other affirmative relief or remedy as a result thereof. In the event of a leveraged buy-out or other take-over of Tenant, Landlord's consent to an assignment of this Lease or subletting of the Demised Premises to the successor entity shall not be deemed to have been unreasonably withheld if said successor entity shall not have a net worth (in the event of a corporate entity, on a market value basis) as certified to by a certified public accountant at least equal to the net worth of Tenant upon the date of execution of this Lease.

Section 11.02. Supplementing the provisions of Section 11.01 of this Lease, provided Tenant is not in default under any of the terms, covenants, conditions and provisions of the Lease, Landlord shall not unreasonably withhold or delay or condition its consent to any proposed assignment of this Lease, or subletting of all or any portion(s) of Demised Premises. Any assignment or transfer of this Lease and any subletting of all or a portion of the Demised Premises shall be subject to Landlord's prior written consent and subject to the terms of all of the sections of this Article 11 and shall be made only if, and shall not be effective until, the assignee or subtenant shall execute, acknowledge and deliver to Landlord a recordable agreement, in form and substance satisfactory to Landlord and counsel for Landlord, whereby the assignee or subtenant shall assume for the benefit of Landlord the obligations and performance of this Lease and agree to be personally bound by and upon all of the covenants, agreements, terms, provisions and conditions hereof on the part of Tenant to be performed or observed, and whereby Tenant (and any guarantor of this Lease or of the Tenant's obligations hereunder) covenants and agrees to remain liable as a primary obligor for the due performance of all of the covenants, agreements, terms, provisions and conditions of this Lease on the part of Tenant to be performed or observed. In the event of any assignment of this Lease or any subletting of all or any portion of the Demised Premises, the obligations of Tenant and any guarantor of this Lease or any guarantor of the obligations of Tenant under this Lease as a primary obligor shall be unaffected and shall remain in full force and effect.

Section 11.03. In the event that Tenant desires to assign this Lease or sublet all or a portion of the Demised Premises, Tenant shall first notify Landlord in writing of its intention, and such notice shall include the information described in the last sentence of Section 11.04 hereof and state the name of the proposed assignee or subtenant, together with its full address and a description of its proposed use (but nothing contained herein shall permit, nor obligate Landlord to permit, a use other than the use permitted by Section 2.01 of this Lease, it being understood that any change in

use shall be subject to Landlord's consent, which Tenant agrees may, notwithstanding anything contained herein to the contrary, be unreasonably withheld). Tenant shall include therewith such financial information as may be available concerning the proposed assignee or subtenant, including without limitation current updated financial statements (which financial information Tenant, and/or the proposed assignee or subtenant shall supplement on demand if required by Landlord). In addition, Tenant shall simultaneously tender a duplicate original of the instrument of assignment or sublease and a termination and surrender agreement in proper form, reasonably satisfactory to counsel for Landlord ("Surrender Agreement") executed in and on behalf of Tenant. Thereafter, Landlord shall have sixty (60) days in which to decide whether to accept a surrender of the Demised Premises or to respond to the notification above, it being understood that during such sixty (60) day period Landlord shall have the right to negotiate with such assignee and/or subtenant, without Landlord incurring any obligation whatsoever to Tenant, for all or a portion of the Demised Premises or such other or greater or lesser area of the Center as Landlord shall determine in its sole discretion. In the event that Landlord shall accept the Surrender Agreement, Landlord shall execute the Surrender Agreement and this Lease shall terminate as of the sixtieth day following the day that Landlord received Tenant's notification ("Surrender Date"), with the same force and effect as if such date were the Expiration Date. Upon the termination of this Lease pursuant hereto, Tenant shall pay all annual minimum rent and additional rent on a pro rata basis for each day prior to the Surrender Date. In the event that any item of additional rent cannot be calculated as of the Surrender Date, Tenant hereby covenants to pay its pro rata share promptly upon being billed therefor and this obligation shall survive the Surrender Date.

Section 11.04. Tenant hereby covenants and agrees to tender to Landlord upon receipt fifty (50%) percent of any annual minimum rent or additional rent or lump sum or installment payment or sum which Tenant shall receive from or on behalf of any assignee(s) or subtenant(s) or any occupant by, through or under Tenant, which is in excess of the annual minimum rent or additional rent payable by Tenant in accordance with the provisions of this Lease (or in the event of a subletting of less than the whole of the Demised Premises, the annual minimum rent or additional rent allocable to that portion of the Demised Premises affected by such sublease) less the actual bona-fide expenses paid by Tenant in connection with such subletting or assignment (e.g. cost of alterations, and brokerage, legal and architectural and engineering fees). At the time of submission of the proposed assignment or sublease to Landlord, Tenant shall certify to Landlord in writing whether or not the assignee or subtenant has agreed to pay any such monies to Tenant or any designee of Tenant other than as specified and set forth in such instruments, and if so Tenant shall certify the amounts and time of payment thereof in reasonable detail.

Section 11.05. Notwithstanding anything to the contrary contained in this Article, Tenant may assign this Lease or sublet any portion of the Demised Premises at any time during the term of this lease, without obtaining Landlord's consent, upon Tenant giving Landlord prior written notice, to (a) another corporation succeeding to substantially all of the assets of Tenant as a result of a consolidation or merger or to a corporation to which all or substantially all of the

assets of Tenant have been sold; (b) a wholly-owned subsidiary corporation; or (c) an affiliated corporation (defined as any corporation whose majority of shares are owned or controlled by the same persons owning or controlling the majority of shares of Tenant); provided: (i) documentation in compliance with Section 11.02 above shall be delivered to Landlord prior to the effective date of such assignment or sublease, and (ii) Tenant shall remain primarily liable under all terms and conditions of this Lease (unless Tenant's corporate existence ends as a matter of law pursuant to such consolidation or merger).

ARTICLE 12. COMMON AREA MAINTENANCE

Section 12.01. As used in this Lease, the term "Common Area Operating Costs" shall include the total cost and expense incurred by Landlord in operating, lighting, striping, maintaining, cleaning, landscaping, repairing (including replacement and resurfacing) managing, signing, equipping and insuring the Common Areas within Lot Nos. 46.24 and 46.25 plus ten (10%) percent of the foregoing costs to cover Landlord's administrative and overhead costs. Such costs and

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expenses shall include, without limitation (including appropriate reserves): cleaning; fire and police protection and general security (Landlord not incurring or assuming any obligation to provide such protection or security or any liability for the failure of the same); repairing and replacing paving; keeping the Common Areas supervised, drained, reasonably free of snow, ice, rubbish and other obstructions, and in a neat, clean, orderly and sanitary condition; the charges for rubbish containers and removal (except that at Landlord's option, Tenant shall be directly responsible for contracting for and for providing (subject to Landlord's approval of the provisions and conditions of the agreement therefor) rubbish containers and removal); the maintenance of any and all fire protection systems servicing Lot Nos. 46.24 and 46.25; the cost of public liability insurance; keeping the Common Areas suitably lighted; maintaining signs (other than Tenant's signs), markers, painted lines delineating parking spaces, and other means and methods of pedestrian and vehicular traffic control; constructing, maintaining and repairing of onsite and offsite traffic controls; maintaining adequate roadways, entrances and exits; maintaining any plantings and landscaped areas; Lot Nos. 46.24 and 46.25 management fees incurred by Landlord, including management fees payable to parties or entities owned or controlled by Landlord or any of them; maintenance and repair of all utilities, utility conduits and storm drainage systems situated within or servicing Lot Nos. 46.24 and 46.25; fees for required licenses and permits; and depreciation of machinery and equipment used in the operation and maintenance of the Common Areas and personal property taxes and other charges incurred in connection with such equipment. The term "Common Areas" shall be defined as all paved areas, driveways, truckways, walkways, and landscaped and planted areas within Lot Nos. 46.24 and 46.25. Landlord shall maintain, light, clean and repair (including snow removal) the Common Areas so that such Common Areas may be used for their intended purposes, and in order to enable Landlord to perform its obligations as aforesaid, Landlord may incur such Common Area Operating Costs as Landlord, in its sole discretion, may determine, in the performance of Landlord's obligations hereunder to maintain the Common Areas.

Section 12.02. During the initial term of this Lease and during any extension term hereof, Tenant shall pay Landlord Tenant's proportionate share of Common Area Operating Costs incurred or expended by Landlord as aforesaid. Such payment shall be made to Landlord in monthly installments on or before the first day of each calendar month, in advance, in an amount estimated by Landlord. Following the expiration of each calendar year during the Term hereof, Landlord shall furnish Tenant with a written statement of the actual amount of Tenant's proportionate share of the Common Area Operating Costs for such year. If the total amount paid by Tenant under this section for any calendar year during the Term shall be less than the actual amount due from Tenant for such year, as shown on such statement, Tenant shall pay to Landlord the difference between the amount paid by Tenant and the actual amount due, such deficiency to be paid within ten (10) days after demand therefor by Landlord; and if the total amount paid by Tenant hereunder for any such calendar year shall exceed such actual amount due from Tenant for such calendar year, such excess shall promptly be applied by Landlord to the next accruing monthly installments of Tenant's proportionate share of Common Area Operating Costs or, at Landlord's option, to any other charges payable by Tenant. For the calendar years in which this Lease commences and terminates, the provisions of this section shall apply, and Tenant's liability for its proportionate share of any Common Area Operating Costs for such years shall be subject to a pro rata adjustment based on the number of days of said calendar years during the Term. Prior to or at the commencement of the Term and from time to time thereafter throughout the Term, Landlord will notify Tenant in writing of Landlord's estimate of Tenant's monthly installments due hereunder. Landlord shall have the right to make special assessments from time to time for extraordinary Common Area Operating Costs and Tenant shall pay any such special assessment within ten (10) days following Landlord's billing therefor. Extraordinary Common Area Operating Costs shall include, without limitation, any charge not anticipated by Landlord in determining Landlord's estimate of Tenant's proportionate share of Common Area Operating Costs for the year in question and any charges, costs and expenses incurred by Landlord which might cause the amounts paid by Tenant pursuant to Landlord's estimate of Tenant's proportionate share of Common Area Operating Costs for the year in question to be less than the amount actually due from Tenant for such year pursuant to this Section 12.02. Tenant's obligations under this section shall survive the expiration of the Term. Tenant's proportionate share of Common Area Operating Costs shall be a fraction, having as its numerator, the number of square feet of floor area within the Demised Premises and as its denominator, the total number of square feet of floor area of all buildings within Lot Nos. 46.24 and 46.25 or, at Landlord's option, the

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portion thereof affected by such cost, including the Demised Premises. Notwithstanding the foregoing provisions of this Article, in the event the obligations of Tenant under this Article 12 are specifically identifiable separate charges relating to Tenant and/or the Demised Premises, then, and in such event, the obligations of Tenant under this Article 12 may, at Landlord's option, be measured and payable in accordance with such separate and specifically identifiable charge and not by the provisions of the preceding sentence.

Section 12.03. Tenant, its concessionaires, officers, employees, and agents may use the Common Areas, subject to such reasonable rules and regulations as Landlord may from time to time impose, including the designation of specific areas in which vehicles owned or operated by Tenant, its concessionaires, officers, employees and agents must be parked. Tenant shall abide by such rules and regulations and cause its concessionaires, officers, employees, agents, customers and invitees to conform thereto. Landlord may, at any time, close temporarily any Common Areas to make repairs or changes therein or to effect construction repairs or changes within Lot Nos.46.24 and 46.25, and Landlord may do such other acts in and to the Common Areas as in its reasonable judgment may be desirable to improve the convenience thereof. In the event that any such temporary closures of the Common Areas shall exceed two (2) business days, Landlord shall use diligent efforts to arrange for appropriate alternate parking for Tenant.

Section 12.04. Notwithstanding anything to the contrary herein contained, Landlord hereby reserves the right (and Tenant hereby consents thereto) to construct or permit the construction, use and maintenance within the Common Areas of Lot Nos. 46.24 and 46.25 including without limitation, the parking areas,

of various commercial type buildings, structures, and appurtenances, and equipment incidental thereto, subject, however, to the provisions of the last sentence of Section 1.01(b).

ARTICLE 13. UTILITIES

Section 13.01. Tenant shall pay, as and when they shall be due and payable, all water charges, taxes, water rates and/or meter charges, sprinkler charges (standby or otherwise), sewer taxes, sewer charges, sewer fees, and sewer rental taxes and charges for utilities, including, without limitation, the charges for gas, electricity, and other utilities furnished to Tenant and consumed in the Demised Premises. Tenant shall heat the Demised Premises whenever the weather shall require. If Landlord, or any property of Landlord, shall be held responsible for any expense covered by this Article, Tenant shall pay Landlord the amount thereof within five (5) days following written request. Landlord shall not be responsible to Tenant for any failure or interruption of any such services, irrespective of the cause thereof, except the negligence or misconduct of Landlord, or Landlord's employees agents, or contractors.

ARTICLE 14. TAXES

Section 14.01. (a) Subject to the reimbursement obligations of Tenant hereinafter set forth, Landlord shall pay during the Term of this Lease, all real estate taxes assessed or imposed upon or respecting the land and improvements within and upon Lot No. 46.24. The term "real estate taxes" for purposes of this Lease shall exclude income, franchise, estate or inheritance taxes levied against Landlord or taxes based upon rental receipts, but shall include any taxes levied in lieu of or as a substitute for real estate taxes. Tenant shall pay to Landlord, as additional rent, at the time and in the manner set forth in Section 14.01 (b), Tenant's proportionate share of such taxes, which proportionate share shall be a fraction having as its numerator the number of square feet of floor area within the Demised Premises and as its denominator, the total number of square feet of floor area of all buildings within Lot 46.24. Notwithstanding the foregoing, if the improvements within the Demised Premises and/or the balance of the improvements or any part thereof upon Lot No. 46.24 shall receive a separate assessment based upon the certification of the Tax Assessor, then the taxes payable by Tenant for such improvements may, at Landlord's option, be based thereon.

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Section 14.01. (b) All amounts payable by Tenant pursuant to this Article shall be paid to Landlord in monthly installments on or before the first day of each calendar month, in advance, in an amount estimated by Landlord; provided, that in the event Landlord is required under any mortgage encumbering Lot No. 46.24 to escrow real estate taxes, Landlord may, but shall not be obligated to, use the amount required to be so escrowed as a basis for its estimate of the monthly installments due from Tenant hereunder. As soon as shall be reasonably practicable following the expiration of each calendar year during the Term, Landlord shall furnish Tenant with a written statement of the actual amount of Tenant's share of the taxes for such year. If the total amount paid by Tenant under this Section for any calendar year during the Term shall be less than the actual amount due from Tenant for such year, as shown on such statement, Tenant shall pay to Landlord the difference between the amount paid by Tenant and the actual amount due, such deficiency to be paid within ten (10) days after demand therefor by Landlord; and if the total amount paid by Tenant hereunder for any such calendar year shall exceed such actual amount due from Tenant for such calendar year, such excess shall be applied by Landlord to the next accruing monthly installments of taxes due from Tenant or, at Landlord's option, to any other charges payable by Tenant. For the calendar years in which this Lease commences and terminates the provisions of this Section shall apply, and Tenant's liability for its share of taxes for such years shall be subject to a pro rata adjustment based on the number of days of said calendar years during the Term. Prior to or at the commencement of the Term and from time to time thereafter throughout the Term, Landlord may notify Tenant in writing of Landlord's estimate of Tenant's monthly installments due hereunder. Landlord shall have the right to make special assessments from time to time for extraordinary real estate taxes and Tenant shall pay any such special assessment within ten (10) days following Landlord's billing therefor. Extraordinary real estate taxes shall include, without limitation, any charge not anticipated by Landlord in determining Landlord's estimate of Tenant's proportionate share of real estate taxes for the year in question and any real estate taxes incurred by Landlord which might cause the amounts paid by Tenant pursuant to Landlord's estimate of Tenant's proportionate share of real estate taxes for the year in question to be less than the amount actually due from Tenant for such year pursuant to this Section 14.01(b). Tenant's obligations under this Section shall survive the expiration of the Term.

Section 14.02. Tenant shall be liable for all taxes on or against property and trade fixtures and equipment placed by Tenant in or about the Demised Premises, and all taxes on Tenant's right to occupy the Demised Premises. If any such taxes are levied against Landlord or Landlord's property, and if Landlord pays same, or if the assessed valuation of Landlord's property is increased by the inclusion therein of a value placed upon such property, and if the Landlord pays the taxes based on such increased assessment, Tenant, upon demand, shall repay to Landlord the taxes so paid by Landlord or the portion of such taxes resulting from such increase in assessment.

ARTICLE 15. REMEDIES OF LANDLORD

Section 15.01. (a) If Tenant shall default in the payment of the annual minimum rental reserved herein, or in the payment of any item of additional rent or other monies due hereunder, or any part of same, and any such default shall continue for more than five (5) days after written notice of such default; or

Section 15.01. (b) If Tenant shall default in the observance of any of the provisions, covenants and conditions of this Lease (other than a default covered by subsection (a) above and other than sections hereof which provide a specific period or date for performance), and such default shall continue for more than twenty (20) days after written notice of such default, or for such other period provided in the relevant section hereof provided, however, that Tenant shall not be in default if Tenant commences to cure such default within said twenty (20) day period and thereafter diligently pursues said cure; or

Section 15.01. (c) If Tenant shall fail to occupy the Demised Premises and open for business at the commencement of the Term of this Lease, as above provided, or if the Demised Premises shall be abandoned, deserted or vacated for a period exceeding three (3) consecutive months, or if Tenant shall sublet the Demised Premises or assign this Lease, except as herein provided, or if Tenant shall

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be in default under any other obligations of Tenant to Landlord of any nature whatsoever, including in connection with any other lease between Tenant and any of the Landlords or between Tenant and any entity in which any partner of Landlord holds an interest; or

Section 15.01. (d) If Tenant or any guarantor of Tenant's obligations hereunder shall make an assignment for the benefit of creditors, or if any such party shall file or have filed against it a petition in bankruptcy, or be adjudicated a bankrupt by any court and such adjudication shall not be vacated within thirty (30) days, or if Tenant or any guarantor of Tenant's obligations hereunder takes the benefit of any insolvency act, or if Tenant or any guarantor of Tenant's obligations hereunder be dissolved voluntarily or involuntarily or have a receiver of its property appointed in any proceedings other than bankruptcy proceedings and such appointment shall not be vacated within thirty (30) days after it has been made, or if any levy, sale or execution of any kind is made upon or of any property of Tenant in the Demised Premises; or

Section 15.01. (e) If, within any period of three hundred and sixty-five consecutive days: (i) Tenant shall default (the "First Default") in any of its obligations under this Lease and notice thereof shall be given to Tenant as provided above; and (ii) Tenant shall default under this Lease for a second time (the "Second Default") (notwithstanding that Tenant may have cured the First Default) and notice thereof shall be given to Tenant as provided above, and (iii) Tenant shall default under this Lease for a third time (the "Third Default") (notwithstanding that Tenant may have cured the First Default and/or the Second Default) and notice thereof shall be given to Tenant as provided above, and the First Default, Second Default and Third Default shall be similar (without limiting the definition of the term "similar", with respect to non-monetary defaults, all monetary defaults shall be deemed to be similar to each other);

then, upon the happening of any one or more of the defaults or events specified above, at the option of Landlord: (1) this Lease and the Term hereof shall wholly cease and terminate, with the same force and effect as though such termination was the date of the expiration of the Term of this Lease, and thereupon, or at any time thereafter, Landlord may re-enter said premises either by force, or otherwise, and have possession of the same and/or may recover possession thereof by summary proceeding, or otherwise (but Tenant shall remain liable to Landlord as hereinafter provided); or (2) Landlord may, without further notice, exercise any remedy available at law or in equity.

Section 15.02. In case of any default, event, re-entry, expiration, termination and/or dispossession by summary proceedings, or otherwise, Tenant shall, nevertheless, remain and continue liable to Landlord in a sum equal to all annual minimum rental and additional rent herein reserved for the balance of the Term herein demised as the same may become due and payable pursuant to the provisions of this Lease. Landlord may repair or alter the Demised Premises in such manner as to Landlord may seem necessary or advisable, and/or let or relet the Demised Premises and any and all parts thereof for the whole or any part of the remainder of the original Term hereof or for a longer period, in Landlord's name, or as the agent of Tenant, and, out of any rent so collected or received, Landlord shall, first, pay to itself, the expense and cost of retaking, repossessing, repairing and/or altering the Demised Premises, and the expense of removing all persons and property therefrom, second, pay to itself, any cost or expense sustained in securing any new tenant or tenants, and third, pay to itself, any balance remaining on account of the liability of Tenant to Landlord for the sum equal to the annual minimum rental and additional rent reserved herein and unpaid by Tenant for the remainder of the Term herein demised. Any entry or re-entry by Landlord, whether had or taken under summary proceedings or otherwise, shall not absolve or discharge Tenant from liability hereunder.

Section 15.03. Should any rent so collected by Landlord after the payment aforesaid be insufficient fully to pay to Landlord a sum equal to all annual minimum rental and additional rent herein reserved, the balance or deficiency shall be paid by Tenant on the rent days herein specified; that is, upon each of such rent days Tenant shall pay to Landlord the amount of the deficiency then existing and Tenant shall be and remain liable for any such deficiency, and the right of Landlord to recover from Tenant the amount thereof, or a sum equal to the amount of all annual minimum rental and additional rent herein reserved if there shall be no reletting, shall survive the issuance of any dispossessory warrant or other termination hereof.

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Section 15.04. Suit or suits for the recovery of such deficiency or damage, or for a sum equal to any installment or installments of annual minimum rental or additional rent hereunder, may be brought by Landlord from time to time at Landlord's election, and nothing herein contained shall be deemed to require Landlord to await the date on which this Lease or the Term hereof would have expired by limitation had there been no such default by Tenant or no such termination or cancellation.

Section 15.05. Tenant hereby expressly waives service of any notice of intention to re-enter subsequent to the giving of the aforesaid notices under Section 15.01 above. Tenant hereby expressly waives any and all right to recover or regain possession of the Demised Premises or to reinstate or to redeem this tenancy or this Lease as is permitted or provided by or under any statute, law, or decision now or hereafter in force and effect.

Section 15.06. Tenant shall reimburse Landlord, within five (5) days following written demand, for any reasonable counsel fees or collection charges actually incurred or expended by Landlord by reason of Tenant's default in the performance of any provision, covenant, or condition of this Lease and any such amounts, at the option of Landlord, may be recovered in the same action or proceeding forming the basis of the default or in another action or proceeding.

Section 15.07. Notwithstanding any other remedy provided for hereunder and without the requirement of notice, except as provided in this Section, if Tenant shall not comply with any of its obligations hereunder, Landlord shall have the right, at Landlord's sole option, at anytime in the event of an emergency or otherwise after ten (10) days notice to Tenant, to cure such breach at Tenant's expense. Tenant shall reimburse Landlord, within ten (10) days following demand, as additional rent, for all costs and expenses incurred by Landlord in curing such breach, together with interest computed thereon at the rate of eighteen (18%) percent per annum or the maximum rate permitted by law, whichever shall be the lesser.

Section 15.08. Notwithstanding anything to the contrary contained in this Lease, if Tenant fails to pay any rent, additional rent or any other money item due hereunder within thirty (30) days after same are due and payable, Landlord shall have the right (in addition to any other rights or remedies of Landlord and without the requirement of any notice) to commence immediate legal proceedings or action for dispossession and damages or Landlord may avail itself of any other remedies at law or in equity and include in such action or proceeding any amounts then due and payable as of the date of the commencement of such action or proceeding. Notwithstanding anything contained in this Lease, if Tenant fails to pay any monetary items due hereunder on the date on which the same are due and payable, a late charge of four ($.04) cents for each ONE ($1.00) DOLLAR so overdue shall become immediately due and payable to the Landlord as damages for failure to make prompt payment and the same shall be considered as additional rent hereunder payable together with the next installment of monthly rent. In addition, all such unpaid monetary items shall bear interest at the maximum rate permitted by law from the date such monies were due until the date on which Landlord shall receive payment.

Section 15.09. The rights and remedies whether herein or elsewhere provided in this Lease shall be cumulative and the exercise of any one right or remedy shall not preclude the exercise of or act as a waiver of any other right or remedy of Landlord hereunder, or which may be existing at law, or in equity, by statute or otherwise.

Section 15.10. Tenant covenants and agrees to give any mortgagee and/or ground lessor of the Center or any portion thereof notice of any default by Landlord under this Lease and such mortgagee and/or ground lessor shall be afforded the right (but shall not have the obligation) to cure any default by Landlord within such reasonable period of time as may be required by such mortgagee and/or ground lessor.

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ARTICLE 16. WAIVER OF TRIAL BY JURY

Section 16.01. It is mutually agreed by and among Landlord, Tenant and any guarantor of the obligations of Tenant hereunder, that the respective parties hereto shall and they hereby do waive trial by jury in any action, proceeding, or counterclaim brought by the parties hereto on any matters whatsoever arising out of or in any way connected with this Lease, the relationship of Landlord and Tenant, Tenant's use or occupancy of the Demised Premises, and/or any claim of injury or damage, and any emergency, summary or statutory remedy. If Landlord commences any summary proceeding, or any other action for collection of rent or additional rent hereunder, Tenant shall not interpose any counterclaim or cross claim of any nature in any such proceeding or action, nor shall Tenant move to consolidate any such claim with any claim being maintained by Landlord.

ARTICLE 17. ACCESS TO PREMISES

Section 17.01. Landlord and its designees shall have the right to enter upon the Demised Premises at all times during normal business hours and upon twenty four (24) hours telephonic notice to Tenant's designated representation at the Demised Premises (except in emergency situations) to inspect and examine same, to make repairs, additions, alterations, or improvements to the Demised Premises, the Building within which the Demised Premises are located or any property owned or controlled by Landlord within such Building. In the case of emergency situations, Landlord may have access to the Demised Premises but shall still be required to give telephonic notice to Tenant's designated representative at the Demised Premises within a reasonable time following Landlord's gaining access to the Demised Premises. Any work performed by Landlord shall be performed in such a manner so as not to interfere with any ongoing experiments being conducted by Tenant at the Demised Premises. Landlord's rights of entry as aforesaid, and the taking of all property into and upon the Demised Premises that may be required in connection therewith, shall not be considered an eviction of Tenant, in whole or in part, constructive or otherwise, and Landlord shall not be liable to Tenant for any expense, damage, or loss or interruption of the business of Tenant by reason thereof, and the rent reserved hereunder shall continue without abatement during the period of any such entry and while such repairs, alterations, improvements or additions are being made. Landlord or Landlord's designees shall have the right to enter the Demised Premises at all times to show the Demised Premises to prospective purchasers, mortgagees or lessees of the Demised Premises, the Building or the Center. During the six month period prior to the expiration of the Term hereof, Landlord may exhibit the Demised Premises to prospective tenants and Landlord may place upon the Demised Premises notices reading, "To Let" or "For Rent", which notices Tenant shall allow to be posted conspicuously without molestation.

ARTICLE 18. NO WAIVER

Section 18.01. No delay or omission of the exercise of any right by either party hereto shall impair any such right or shall be construed as a waiver of any default or as acquiescence therein. One or more waivers of any provision, covenant, or condition of this Lease by either party shall not be construed by the other party as a waiver of a subsequent breach of any other or the same provisions, covenant, or condition. No requirements whatsoever of this Lease shall be deemed waived or varied because of either party's failure or delay in taking advantage of any default, and Landlord's acceptance of any payment from Tenant with actual or constructive knowledge of any default shall not constitute a waiver of Landlord's rights in respect to such default, nor of any subsequent or continued breach of any such default or any other requirement of this Lease.

Section 18.02. No payment by Tenant or receipt and acceptance by Landlord of a lesser amount than the rent or other sum stipulated to be paid or reserved shall be deemed an accord and satisfaction or a modification or waiver of any rights or obligations or liabilities hereunder notwithstanding any statement, written or oral, accompanying such payment, or by way of endorsement or otherwise; and Landlord may accept any such payment whether by check, draft or other means whatsoever without prejudice to Landlord's right to recover the balance owing, or to

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pursue any other remedy in this Lease or at law or in equity provided. Landlord may apply such payment to any sums then due and payable by Tenant to Landlord as Landlord shall determine in its sole discretion. Landlord may, at Landlord's option, accept payment of rent or any other charge hereunder from any person or entity other than the Tenant named herein and the same shall not constitute a recognition by Landlord of, or vest in said person or entity, any rights hereunder.

ARTICLE 19. REQUIREMENTS OF LAW; INSURANCE REQUIREMENTS

Section 19.01. In Tenant's performance of its rights and obligations under this Lease, including without limitation, any pre-term right, obligation or entry into the Demised Premises, Tenant covenants and agrees to comply with all laws, orders, and regulations of federal, state, city, county, governmental and municipal authorities, fire insurance rating organizations and fire insurance underwriters, and insurance companies issuing coverage respecting the Demised Premises and Tenant shall make all alterations or installations necessary to comply therewith. Tenant shall secure all permits or approvals necessary to operate its business within the Demised Premises and shall only operate its business within the Demised Premises in compliance with all laws, orders and regulations of federal, state, city and county, governmental and municipal authorities, fire insurance rating organizations and fire insurance underwriters, and insurance companies issuing coverage respecting the Demised Premises.

Section 19.02. Tenant shall not use or occupy the Demised Premises or do or permit anything to be done therein in any manner which shall make it impossible for Landlord and/or Tenant to obtain at standard rates any insurance required or desired, or which will invalidate or increase the cost to Landlord of any insurance. Landlord hereby acknowledges that Tenant shall be permitted to use and occupy the Demised Premises for the chemical and biological laboratory research contemplated to be conducted in the Demised Premises, provide same is conducted in accordance with industry standards and the requirements of all applicable governmental authorities.

Section 19.03. If, by reason of Tenant's failure to comply with the provisions of Section 19.01 above, or if, by reason of any act or failure to act of Tenant, its agents, servants, contractors, employees or licensees, or if, by reason of the use of the Demised Premises, the fire insurance rates applicable to the Demised Premises, or of the Building or any other premises in said Building, shall be increased above the rate applicable to the occupancy permitted hereunder, Tenant shall pay to Landlord, within three (3) days following demand, the amount of additional premium for fire insurance payable by reason thereof.

Section 19.04. No abatement, diminution, or reduction in annual minimum rental or any sums constituting additional rent shall be claimed by or allowed to Tenant for any inconvenience or interruption, cessation or loss of business caused directly or indirectly, by any present or future laws, ordinances, rules or regulations, requirements or orders of federal, state, county, township or municipal governments or any other lawful authority whatsoever, or by priorities, rationing, or curtailment of labor or materials, or by war, civil commotion, strikes or riots, or any manner or thing resulting therefrom, or by any other cause or causes beyond the control of Landlord, nor shall this Lease be affected by any such causes.

ARTICLE 20. SIGNS

Section 20.01. Tenant shall not place, install or maintain any sign upon or outside the Demised Premises or in the Center until approved by Landlord, nor shall Tenant place, install or maintain any sign within one-half mile of the Center; nor shall Tenant place, install or maintain any awning, canopy, aerial, antenna or the like in or upon the Demised Premises, the Building or the Center. Landlord's approval of Tenant's signs shall not be unreasonably withheld, conditioned or delayed. Any sign must conform to all applicable rules, regulations, codes and directives of governmental agencies having jurisdiction, and Tenant shall, at its expense, apply for and obtain all permits necessary in connection therewith. If Landlord shall submit to Tenant a general sign criteria or specification, Tenant shall comply therewith. Tenant shall be solely responsible for all maintenance and repairs respecting its signs.

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ARTICLE 21. TENANT'S ADDITIONAL COVENANTS

Section 21.01. Tenant covenants and agrees for itself, its officers, employees, contractors, agents, servants, licensees, invitees, subtenants, concessionaires, and all others doing business with Tenant (hereinafter for the purposes of this Article, collectively referred to as "Tenant") that:

(a) Tenant shall execute such further assurances and/or guarantees which are stated by Landlord as intended to be necessary to secure the approval of Tenant's credit rating;

(b) Tenant shall not encumber or obstruct the Center or sidewalks in and about the Demised Premises;

(c) Tenant shall not display, advertise or sell its products or goods in the Common Areas of the Center or sidewalk in and about the Demised Premises;

(d) Intentionally omitted.

(e) Tenant shall not cause or permit trash, refuse, dirt or other rubbish to accumulate on the Demised Premises or in the Center and shall cause same to be promptly removed;

(f) Tenant shall not injure, overload, deface, commit waste or otherwise harm the Demised Premises or any part thereof;

(g) Tenant shall not commit any nuisance;

(h) Tenant shall not permit the emission from the Demised Premises of any objectionable noise or odor. Normal laboratory practices conducted in accordance with industry standards and the requirements of governmental authorities shall be excepted from the foregoing;

(i) Tenant shall not burn any trash, rubbish, dirt or refuse within the Center;

(j) Tenant shall use the Demised Premises only for business and commercial purposes (subject to the provisions of Article 2 hereof) and Tenant shall not use, allow or permit any industrial, manufacturing or processing activities within the Demised Premises, except as may be expressly permitted by Section 2.01 of this Lease;

(k) Tenant shall conform and comply with all nondiscriminatory and uniformly applicable rules and regulations which Landlord may promulgate for the management and use of the Center;

(l) Tenant shall not use any advertising medium that may constitute a nuisance, such as loudspeakers, sound amplifiers or phonographs, in a manner to be heard outside the Demised Premises;

(m) Intentionally omitted.

(n) Tenant shall not place a load on any floor of the Demised Premises exceeding the floor load per square foot which such floor was designed to carry;

(o) Tenant shall not install, operate or maintain in the. Demised Premises any electrical equipment which will overload the electrical system therein or any part thereof beyond the capacity for proper and safe operation, as determined by Landlord, in relation to the overall system and requirements for electricity in the Building;

(p) Tenant shall not install, operate, or maintain any electrical equipment in the Demised Premises which does not bear underwriters approval; and

(q) No portion of the Demised Premises shall be used or occupied for the sale, dispensing, storage or display of food, foodstuffs, or food products for consumption on or off the Demised

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Premises, provided that the foregoing shall not prohibit the use and occupancy of the Demised Premises as permitted by Section 2.01 hereof, and further provided that the foregoing shall not prohibit vending machines, refrigerators, microwave ovens or other food service areas maintained by Tenant for the use of Tenant's employees and visitors.

ARTICLE 22. EASEMENTS FOR UTILITIES

Section 22.01. Landlord or its designee shall have the right and Tenant shall permit Landlord or its designee to erect, use, maintain and repair pipes, cables, conduits, plumbing, vents and wires in, to and through the Demised Premises as and to the extent that Landlord may now or hereafter deem necessary or appropriate for the use or proper operation and maintenance of the Demised Premises, or the Building or any other portion of the Center. Landlord's rights under this Article shall be exercised, as far as practicable, in such manner as to avoid unreasonable interference with Tenant's occupancy of the Demised Premises.

ARTICLE 23. CONSENTS AND APPROVALS

Section 23.01. With respect to any provision of this Lease providing that Landlord shall not unreasonably withhold or unreasonably delay any consent or any approval, Tenant, in no event, shall be entitled to make, nor shall Tenant make, any claim for, and Tenant hereby waives any claim for money damages; nor shall Tenant claim any money damages by way of setoff, counterclaim or defense, based upon any claim or assertion by Tenant that Landlord has unreasonably withheld or unreasonably delayed any consent or approval; but Tenant's sole remedy shall be an action or proceeding to enforce any such provision, or for specific performance, injunction or declaratory judgment.

ARTICLE 24. CONTROL OF TENANT

Section 24.01. If Tenant hereunder is a corporation (other than a corporation whose shares are regularly and publicly traded on a duly recognized stock exchange), Tenant represents that ownership and power to vote its entire outstanding capital stock is vested in the officers executing this Lease or the members of their immediate families. If there shall be any change in the ownership of and/or power to vote the majority of said outstanding capital stock, without the prior written consent of Landlord, then, and in such event, Landlord shall have the option to terminate this Lease upon not less than thirty (30) days notice to Tenant.

ARTICLE 25. END OF TERM HOLDOVER

Section 25.01. If the last day of the Term falls on a Sunday, or legal holiday, this Lease shall expire on the business day immediately following. Upon the expiration or other termination of the Term of this Lease, Tenant shall quit and surrender to Landlord the Demised Premises, together with all buildings and improvements thereon, "broom-clean" and in good order and condition, ordinary wear and tear and damage by the elements excepted, and Tenant shall thereupon remove all property of Tenant which shall be deemed to include, without limitation, the removal of all of Tenant's laboratory equipment and fixtures such as laboratory hoods and benches, including the capping of all utilities for such equipment, but shall not include the removal of the acid neutralization pit and tank. In the event Tenant fails to remove such property, Landlord may cause all of the said property to be removed, stored and/or disposed of at the expense of Tenant. Tenant shall pay all costs and expenses thereby incurred. Any property not so removed shall be deemed to have been abandoned by Tenant and may be retained or disposed of by Landlord as Landlord, in its sole discretion, shall determine and Tenant hereby releases Landlord from all claims for loss or damage to such property arising out of such retention or disposition thereof. Tenant's obligations under this Article shall survive the expiration or other termination of the Term.

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Section 25.02. If Tenant remains in possession of the Demised Premises at the expiration or earlier termination of the Term hereof, Tenant, at Landlord's option, shall be deemed to be occupying the Demised Premises as a tenant from month to month, at a monthly rental equal to the greater of (i) one and one-half times the sum of the monthly installment of annual minimum rent payable during the last month of the Term hereof or (ii) twice the sum of the then prevailing market rate rent, as determined by Landlord at its sole and absolute discretion, plus all additional rent coming due hereunder. Acceptance by Landlord of rent after the expiration or earlier termination of the Term hereof shall not constitute a consent to a month-to- month tenancy or result in a renewal. In the event of such holdover, Tenant's occupancy of the Demised Premises, except as aforesaid, shall be subject to all other conditions, provisions and obligations of this Lease, but only insofar as the same are applicable to a month to month tenancy. Such month to month tenancy shall be terminable by Landlord upon one (1) month's notice to Tenant, and if Landlord shall give such notice, Tenant shall quit and surrender the Demised Premises to Landlord as above provided. In the event that (a) Tenant shall remain in possession of the Demised Premises at the expiration or earlier termination of the Term hereof and Landlord shall not have elected to deem Tenant to be occupying the Demised Premises as a tenant from month-to-month or (b) Landlord shall terminate any month-to-month tenancy of the Demised Premises and Tenant shall fail to quit and surrender the Demised Premises to Landlord upon the termination date as above provided, then, in either such event, Tenant shall be liable to Landlord for all losses, damages, claims, costs and/or expenses incurred by Landlord by reason of Tenant's failure to deliver timely possession of the Demised Premises to Landlord, including, without limitation, any consequential and incidental damages so incurred by Landlord, including, without limitation, any losses, damages, claims, costs and/or expenses incurred in connection with or arising from the inability of Landlord to lease and deliver possession of the Demised Premises, or any portion thereof, to any third party and/or the termination or cancellation of any lease of the Demised Premises, or any portion thereof to any third party.

Section 25.03. Notwithstanding anything to the contrary contained in this Lease, if Landlord shall be unable to provide possession of the Demised Premises because of the holding-over or retention of possession of any prior tenant, undertenant or occupants, or for any other reason whatsoever, Landlord shall not be subject to any liability for the failure to give possession on the date herein provided, if any, and the validity of this Lease shall not be impaired under such circumstances, but the Term shall be extended proportionately until after Landlord shall have given Tenant written notice that the Demised Premises are ready for Tenant's occupancy. If permission is given to Tenant to enter into possession of the Demised Premises or to occupy premises other than the Demised Premises prior to the date specified as the commencement of the Term, Tenant covenants and agrees that such occupancy shall be deemed to be under all of the terms, covenants, conditions and provisions of this Lease.

ARTICLE 26. AUTHORITY TO EXECUTE

Section 26.01. Landlord and Tenant do hereby respectively represent to the other that it has the capacity to enter into this Agreement.

ARTICLE 27. NOTICES

Section 27.01. All notices to be given pursuant to this Lease shall be in writing and sent by prepaid certified or registered U.S. mail, return receipt requested, or by a recognized overnight courier service which requires acknowledgment of receipt of delivery from addressee, to the address of the parties below specified or at such other address as may be given by written notice in the manner prescribed in this paragraph. Landlord's address for notice shall be c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577. Tenant's address for notices given prior to the Commencement Date shall be the address first set forth above for Tenant. Tenant's address for notices given on or subsequent to the Commencement Date shall be the address of the Demised Premises. Notice shall be deemed to be given upon delivery to the U.S. Postal Service or recognized overnight courier service. Unless Landlord shall otherwise inform Tenant in writing to the contrary, any notice given to Tenant by National Realty & Development Corp. shall, for the purposes of this Lease, be deemed to be a notice given to Tenant by Landlord.

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ARTICLE 28. BROKER

Section 28.01. Tenant covenants, warrants and represents that it has dealt with no broker except Colliers Houston & Co., 200 Cottontail Lane, Somerset, New Jersey 08873 ("Colliers/Houston") respecting this Lease and that no conversations, correspondence or negotiations were had with any broker except with said Colliers/Houston concerning the renting or leasing of the Demised Premises. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations with any broker except said Colliers/Houston. Landlord shall pay any commissions owing to said Colliers/Houston in accordance with separate agreement.

ARTICLE 29. MEMORANDUM OF LEASE

Section 29.01. Tenant agrees not to record this Lease. The parties agree, upon request of either, to execute, in recordable form, a short form lease entitled "Memorandum of Lease", it being the intention of the parties that this Lease will not be recorded, but only a memorandum thereof. Such short form lease shall contain those provisions of this Lease as shall be desired in the reasonable discretion of counsel for the parties hereto, provided that in no event shall such short form lease contain any provisions relevant to the annual minimum rent and/or additional rent payable under this Lease.

ARTICLE 30. AIR AND WATER POLLUTION

Section 30.01(a). Tenant hereby indemnifies and saves Landlord harmless against any claim, damage, liability, costs, penalties or fines which the Landlord may suffer as a result of air, land or water pollution caused by Tenant in its use or occupancy or manner of use or occupancy of the Demised Premises or in its storage, handling, possession, transportation and/or disposal of any Hazardous Waste or Hazardous Substance (as such terms are hereafter defined) within or about the Demised Premises. Tenant covenants and agrees to notify Landlord immediately of any claim or notice served upon it with respect to any such claim that Tenant is causing air, land or water pollution; and Tenant, in any event, will take immediate steps to halt, remedy and cure any pollution of air, land or water caused by Tenant by its use of the Demised Premises, at its sole cost and expense.

Section 30.01(b). Landlord hereby represents to Tenant that Landlord has not received any notice or directive, and is not aware of any claim, from any environmental agency having jurisdiction over the Demised Premises regarding any violation of the Environmental Statutes at the Demised Premises. Landlord agrees to indemnify and hold the Tenant harmless from and against any and all claims brought by any such environmental agency relating to the Demised Premises proven to have arisen prior to the date that Landlord delivers possession of the Demised Premises to Tenant. In no event shall Tenant be held responsible for the actions of the prior occupant of the Demised Premises.

Section 30.02. (a) Tenant shall comply with all state and federal environmental laws, including the Spill Compensation and Control Act ("SCCA") (N.J.S.A. 58:10-23.11 et seq.) and Industrial Site Recovery Act ("ISRA") (N.J.S.A. 13:1K-6 et seq.) as the same may have been or may hereafter be amended (collectively, the "Environmental Statutes") as the same may relate to Tenant's use and occupancy or manner of use and occupancy of the Demised Premises or any act or failure to act of Tenant. Tenant shall supply Landlord on demand with any information Landlord may require in order to enable Landlord to comply with the Environmental Statutes, including, without limitation, ISRA, whether upon the transfer of title or closing of operations at the Demised Premises, or for any reason whatsoever.

Section 30.02. (b) Tenant shall not use the Demised Premises for the purpose of refining,

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producing, storing, handling, transferring, processing or transporting said "Hazardous Substances", as such term is defined in N.J.S.A. 5B:10-23.llb(k) of the New Jersey Spill Compensation and Control Act (N.J.S.A. 58:10-23.11 et seq.), except in *dé minimus* quantities in strict compliance with the requirements of all applicable governmental authorities.

Section 30.02. (c) Tenant shall not use the Demised Premises to generate, manufacture, refine, transport, treat, store, handle or dispose of "Hazardous Substances", or "Hazardous Wastes", as such terms are defined in N.J.A.C. 7:1-3.3.

Section 30.02. (d) Tenant shall not cause or permit to exist, as a result of an intentional or unintentional action or omission on its part, a releasing, spilling, leaking, pumping, emitting, pouring, emptying or dumping of a "Hazardous Substance", as such term is defined in N.J.S.A. 58:10-23.llb(k) into waters of the State of New Jersey or onto the lands from which it might flow or drain into said waters, or into waters outside the jurisdiction of the State of New Jersey where damage may result to the lands, waters, fish, shellfish, wildlife, biota, air and other resources owned, managed, held in trust or otherwise controlled by the State of New Jersey. Landlord acknowledges that proper disposal of a Hazardous Substance in accordance with the requirements of all applicable governmental authorities shall not be a violation of this Section 30.02 (d).

Section 30.02. (e) Tenant shall not use the Demised Premises as a “Major Facility”, as such term is defined in N.J.S.A. 58:10-23.lb(1).

Section 30.02. (f) Tenant shall not install nor permit to be installed in the Demised Premises friable asbestos or any substance containing asbestos and deemed hazardous by federal or state regulations respecting such material. Landlord acknowledges that the Demised Premises contains asbestos and Tenant shall not be responsible in any manner therefor.

Section 30.03. Tenant represents that Tenant has not received a summons, citation, directive, letter or other communication, written or oral, from the New Jersey Department of Environmental Protection concerning any intentional or unintentional action or omission on Tenant’s part resulting in the releasing, spilling, leaking, pumping, pouring, emitting, emptying or dumping of “Hazardous Substances”, as such term is defined in N.J.S.A. 58:10-23.llb(k), into the waters or onto the lands of the State of New Jersey, or into the waters outside the jurisdiction of the State of New Jersey resulting in damage to the lands, waters, fish, shellfish, wildlife, biota, air and other resources owned, managed, held in trust or otherwise controlled by the State of New Jersey.

Section 30.04. (a) In the event that Tenant does not expeditiously proceed with any compliance required by any State or Federal authority under the Environmental Statutes, Landlord may elect to undertake such compliance in order to protect its interest in the Demised Premises. Any monies expended by Landlord in efforts to comply with any environmental statute (including but not limited to: the costs of hiring consultants, undertaking sampling and testing, performing any cleanup necessary or useful in the compliance process and attorney’s fees), together with interest at the maximum rate permitted by law, will be added to and payable with the next payment of annual minimum rental due from Tenant, or will be payable on demand of Landlord.

Section 30.04. (b) Tenant will provide Landlord with all information as to the use or manner of use of the Demised Premises by Tenant, and an environmental audit of the Demised Premises which is designed to describe any materials on the Demised Premises which would require a filing and/or any disclosure under the Environmental Statutes in the event of any transfer or closure, or which would require remedial action under any other Environmental Statutes.

Section 30.04. (c) In the event that Tenant receives notice from the Department of Environmental Protection or any other governmental authority or bureau having or asserting jurisdiction thereover under SCCA of a discharge on or about the Demised Premises, or any other notice of violation of the Environmental Statutes or any alleged or claimed violation thereof, Tenant will immediately send a copy of such notice to Landlord and Tenant will promptly proceed to remedy the condition described in the notice. Tenant shall take all action necessary to ensure that the SCCA administrator does not spend Spill Fund monies to clean up the site. In the event that the

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SCCA administrator should spend money cleaning up property owned by Landlord due to Tenant’s use or occupancy or manner of use or occupancy of the Demised Premises or the act or failure to act of Tenant, and/or a lien is imposed on the Demised Premises or any portion of the parcel of which it forms a part or any property of Landlord, Landlord may take such actions as it deems necessary to remove such lien, including satisfaction thereof, or may require it to be bonded by Tenant, and Tenant agrees to defend, indemnify and hold Landlord free and harmless from and against all loss, costs, damage and expense (including attorney’s fees and costs) Landlord may sustain by reason of the assertion against Landlord by any party of any claim in connection therewith. Landlord may demand such security, in amounts and types which it deems appropriate in its sole discretion, for the purpose of protecting its property from any such lien or to guarantee cleanup.

ARTICLE 31. SUBDIVISION

Section 31.01. Landlord shall have the right at any time during the Term or any extension term hereof, and Tenant hereby consents thereto, to subdivide Lot No. 46.24 into such additional lot or lots as Landlord may in its sole discretion elect and/or to expand Lot No. 46.24 as Landlord may in its sole discretion elect, provided that the whole of the Building shall remain entirely within one such subdivision. Notwithstanding anything contained in this Lease to the contrary, in the event of any such subdivision or expansion of Lot No. 46.24 by Landlord then, at Landlord’s option, (i) references in this Lease to Lot No. 46.24 may be deemed to be to the original (pre-subdivision or pre-expansion) Lot No. 46.24 or any portion(s) thereof of which the Demised Premises forms a part, and (ii) in calculating Tenant’s proportionate share(s), Landlord may use as the denominator of the fraction(s) representing Tenant’s proportionate share(s) the building(s) or portions thereof within said original Lot No. 46.24 or any portion(s) thereof of which the Demised Premises forms a part. In the event of such subdivision or expansion, Tenant agrees to execute an agreement in recordable form setting forth the description of Lot No. 46.24 as so subdivided or expanded and as renamed and/or renumbered.

ARTICLE 32. THERE IS NO ARTICLE 32 IN THIS LEASE

ARTICLE 33. FINANCING REQUIREMENTS

Section 33.01. Landlord intends to procure the funds for construction of the Center from one or more lenders. If any such lender disapproves the credit rating of Tenant for purposes of such financing, or if any such lender shall require or suggest changes in this Lease as a condition of its approval of this Lease for such financing and if within ten (10) days following notice from Landlord (a) Tenant fails to execute such further assurances and/or guarantees which are stated by Landlord as intended to be necessary to secure the approval of Tenant’s credit rating, or (b) if Tenant fails to execute, acknowledge and deliver any amendments to this Lease setting forth the changes which are stated by Landlord to be needed in connection with the approval of this Lease for purposes of such financing, or if, for any reason, other than Landlord’s willful default, such financing in amount and interest rate satisfactory to Landlord cannot be obtained, Landlord may terminate this Lease at any time prior to Landlord’s giving of notice of substantial completion. If this Lease be terminated, it shall become null and void and the parties shall be automatically released, as of the date of termination, from any and all liability, rights or obligations hereunder, except that Landlord shall return any security deposited by Tenant hereunder.

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ARTICLE 34. RELATIONSHIP OF PARTIES

Section 34.01. Nothing herein contained shall be deemed or construed by the parties hereto, nor by any third party, as constituting the Landlord a partner of Tenant in the conduct of Tenant's business, or as creating the relationship of principal and agent or joint venturers between the parties hereto, it being the intention of the parties hereto that the relationship between them is and shall at all times be and remain that of Landlord and Tenant only. Tenant agrees upon the demand of Landlord to deliver to Landlord and any mortgagee of Landlord the most recently available financial statements of Tenant and any guarantor of this Lease, certified to by a certified public accountant, and updated to the extent reasonably requested by Landlord or any such mortgagee.

ARTICLE 35. CAPTIONS

Section 35.01. The Article captions contained herein are for convenience only and do not define, limit, or construe the contents of such Articles and are in no way to be construed as a part of this Lease.

ARTICLE 36. DEFINITIONS

Section 36.01. Words of any gender used in this Lease shall be held to include any other gender, and words in the singular number shall be held to include the plural, when the sense requires.

Section 36.02. If any provision of this Lease or the application thereof to any person or circumstances shall, to any extent, be invalid or unenforceable, the remainder of this Lease, or the application of such provision to persons or circumstances other than those as to which it is held invalid or unenforceable, shall not be affected thereby and each provision of this Lease shall be valid and be enforced to the fullest extent permitted by law.

ARTICLE 37. ENTIRE AGREEMENT

Section 37.01. This instrument of Lease contains the entire and only agreement between the parties concerning the Demised Premises. No prior oral or written statements or representation, if any, of any party hereto or any representative of a party hereto, not contained in this instrument, shall have any force or effect. This Lease shall not be modified in any way, except by a writing executed by Landlord and Tenant. No oral agreement or representations shall be deemed to constitute a lease other than this agreement. This agreement shall not be binding until it shall have been executed and delivered by Landlord and Tenant. The submission of this Lease to Tenant prior to its execution by Landlord shall not be an offer to lease.

ARTICLE 38. SUCCESSORS IN INTEREST

Section 38.01. All provisions herein contained shall bind and inure to the benefit of the respective parties hereto, their heirs, personal representatives, successors and assigns, as the case may be. In the event Landlord or any successor-lessor (owner) of the Demised Premises shall convey or otherwise dispose of the Demised Premises and/or the Center and/or the Tax Lot of which the Demised Premises forms a part, all liabilities and obligations of Landlord or such successor-lessor (owner), as Landlord under this Lease shall terminate upon such conveyance or disposal.

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Section 38.02. If Landlord, or any successor in interest to Landlord, shall be an individual, joint venture, tenancy-in-common, trustee, trust, estate, executor, conservator, personal representative, limited liability company, limited liability partnership, partnership, general or limited, firm, company or corporation, there shall be no personal liability on the part of such individual, trustee, executor, conservator or personal representative or on the part of any members, managers, partners, directors, officers and/or shareholders of such joint venture, tenancy-in-common, trustee, trust, estate, executor, conservator, personal representative, limited liability company, limited liability partnership, partnership, general or limited, firm, company or corporation or on the part of such joint venture, tenancy-in-common, trustee, trust, estate, executor, personal representative, limited liability company, limited liability partnership, partnership, general or limited, firm, company, or corporation as to any of the provisions, covenants or conditions of this Lease, Tenant hereby acknowledges that it shall look solely to the real property interest of Landlord in Lot No. 46.24 (or, in the event of a subdivision of said Lot, such subdivided portion thereof which includes the Demised Premises) for the satisfaction or assertion of any claims, rights and remedies of Tenant against Landlord, in the event of breach by Landlord of any of the terms, provisions, covenants or conditions of this Lease.

ARTICLE 39. SECURITY

Section 39.01. Tenant has delivered to Landlord a letter of credit in the amount of TWO HUNDRED SEVENTEEN THOUSAND AND 00/100 ($217,000.00) DOLLARS as security for the faithful performance and observance by Tenant of the terms, provisions, covenants and conditions of this Lease, including without limitation the completion of the Tenant Allowance Work and the Tenant Improvements pursuant to the provisions of Article 5 of this Lease. Landlord and Tenant acknowledge and agree that upon completion of the Tenant Improvements and delivery to Landlord of the documentation required pursuant to Subparagraph (C) of Section 5.05 of this Lease, said $217,000.00 Letter of Credit shall be reduced to the amount of $46,110.00, which amount shall thereafter serve as the security deposit under this Lease. At any time during which Landlord shall be holding a letter of credit as the security deposit under this Lease, Tenant shall have the right to substitute a cash deposit for said letter of credit by delivering to Landlord a check in the amount of the letter of credit then being held by the Landlord. Upon receipt and clearance of the funds, Landlord shall return the letter of credit to Tenant for cancellation. Thereafter, all references herein to the security deposit herein shall be deemed to refer to such cash security deposit.

Upon delivery of this Lease to Landlord as executed by Tenant, Tenant shall deliver to Landlord an irrevocable, unconditional Letter of Credit from Fleet Bank (or another money center bank reasonably acceptable to Landlord), in the form annexed hereto as Exhibit C in the amount of $217,000.00, which letter of credit shall serve as security for the faithful performance and observance by Tenant of the terms, provisions, covenants and conditions of this Lease. Said letter of credit shall name National Realty & Development Corp. as sole beneficiary and shall expire on the Expiration Date hereof; provided, however, said letter of credit may provide that it will expire prior to the Expiration Date (but in no event prior to the one (l) year anniversary of the Commencement Date) if said letter of credit is renewed by Tenant, without amendment, and evidence of such renewal is delivered to Landlord prior to that date which is thirty (30) days prior to the expiration date thereof. The letter of credit shall provide that partial drawings shall be permitted. If, for any reason, such letter of credit shall expire without National Realty & Development Corp. (as agent of Landlord) having drawn thereon for any reason, including, without limitation, the inadvertent failure to do so by National Realty & Development Corp., then Tenant shall deliver to National Realty & Development Corp. a replacement of such letter of credit or a cash deposit to bring the security deposit required hereunder to the appropriate balance. Said letter of credit shall specifically provide that Landlord and National Realty & Development Corp. will receive not less than forty-five (45) days written notice of the election of the issuing bank to not renew the same. Whether or not Landlord

or National Realty & Development Corp. shall receive notice of cancellation or non-renewal of the letter of credit, Tenant shall deliver to National Realty & Development Corp. a replacement of such letter of credit prior to that date which is thirty (30) days prior to the cancellation date, expiration date or non-renewal date of the letter of credit. Tenant's failure to deliver evidence of the renewal of the letter of credit or a replacement letter of credit as aforesaid shall, in either case, be deemed a default under this Lease, and without further notice,

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National Realty & Development Corp. shall be entitled to draw upon the expiring letter of credit in the entire amount thereof.

In the event Tenant defaults in respect of any of the provisions, covenants or conditions of this Lease, including, but not limited to, defaults in the payment of annual minimum rent or additional rent, beyond the applicable notice and cure periods provided for herein, or in the event that that Tenant has vacated, abandoned or deserted the performance of the Tenant Improvements or is not diligently pursuing the same to completion or Tenant has failed to pay for the furnishings, installation or construction of Tenant Improvements (including the Tenant Allowance Work) or any portion thereof, or in the event that Tenant has failed to complete the Tenant Improvements by the Tenant Improvements Completion Date, then National Realty & Development Corp. may, on Landlord's behalf, from time to time draw upon the security deposit and use, apply, or retain the whole or any part thereof to the extent required for the payment of any annual minimum rent (including payment of annual minimum rental previously abated as set forth in Section 5.05 of this Lease) and additional rent or any other sum as to which Tenant is in default or for any sum which Landlord may expend or may be required to expend by reason of Tenant's default, beyond the applicable notice and cure periods provided herein, in respect of any of the provisions, covenants and conditions of this Lease, including, but not limited to, reasonable counsel fees and other collection charges, or with respect to any damages or deficiency in the re-letting, repairing or altering of the Demised Premises, whether such damages or deficiency accrued before or after summary proceedings or re-entry by Landlord, or in connection with the removal of the Installations (hereinafter defined) (the amount which National Realty & Development Corp. may draw determined as set forth in this sentence is hereinafter referred to as the "default amount").

In the event National Realty & Development Corp. (as agent of Landlord) shall draw upon a letter of credit deposited as a security deposit hereunder and the amount drawn by National Realty & Development Corp. shall be in excess of the default amount, the excess shall be held by in a non-interest-bearing account at a commercial bank or financial institution as a security deposit hereunder to be used for the purposes set forth herein. After the expiration of the Lease, and after delivery of entire possession of the Demised Premises to Landlord, and after applying or retaining any portion of the security required to cure any and all defaults by Tenant under this Lease, the letter of credit and the cash security deposit, if any, then held by Landlord shall be returned to Tenant without interest. If, due to Tenant's default hereunder, Landlord shall be entitled to apply or retain any portion of said security, Tenant shall, within five (5) days following demand, secure for the sole benefit of Landlord, a new or additional letter of credit naming Tenant as beneficiary and complying with the requirements set forth herein or deliver to Landlord a cash security deposit sufficient to comply with this Section, including the required amount. Tenant shall not assign or encumber the security deposited hereunder and neither Landlord or its successors or assigns shall be bound by any such assignment or encumbrance. In the absence of evidence satisfactory to Landlord of any assignment of the right to receive the security, or the remaining portion thereof, Landlord may return the security to the original tenant regardless of any number of assignments of the Lease itself. In the event of a sale of the Demised Premises or larger premises of which the Demised Premises form a part, Landlord shall have the right to transfer the cash security and the beneficiary rights under any letter of credit to the purchaser who shall hold the same for the benefit of Tenant in accordance with the terms of this Lease, and Landlord and National Realty & Development Corp., after giving notice to Tenant, shall be deemed released by Tenant from all liability for the return of such security and Tenant shall look solely to the new owner for the release or the return thereof. Tenant shall, upon request, deliver confirmation of said transfer of beneficiary rights and a replacement letter of credit naming the transferee as beneficiary if necessary or if requested. Landlord agrees to return any letter of credit it is then holding with respect to this Lease to the issuing bank if required by the issuing bank to receive a replacement letter of credit. No holder of any mortgage upon the Demised Premises or the larger property of which the Demised Premises forms a part shall be responsible in connection with the security deposited hereunder unless such mortgagee shall have in fact received such security or be named beneficiary thereof and acknowledged such receipt or beneficiary status in writing to Tenant. In the event of a foreclosure of the Demised Premises, or the larger property of which the Demised Premises forms a part, Tenant shall, on demand of mortgagee, reissue the letter of credit in compliance with this Section, naming the mortgagee, or such other party as may be designated by mortgagee, as the sole beneficiary. Tenant acknowledges that Tenant is to perform

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certain obligations under this Lease prior to the Commencement Date of the term of this Lease and that the security deposit may be applied by Landlord (or by National Realty & Development Corp. on behalf of Landlord) in the event Tenant shall default under any such obligations beyond any applicable notice and cure periods notwithstanding that the Commencement Date may not yet have occurred.

ARTICLE 40. EXTENSION OPTION(S)

Section 40.01. Tenant shall have the option, provided it is not in default hereunder, to extend the Term for TWO (2) successive additional term of FIVE (5) years each, upon the same terms and conditions as provided herein, except that the annual minimum rental during said extension period shall be as provided below, and except that Tenant shall have no further extension options. Tenant shall give written notice to Landlord not less than twelve (12) months prior to the last day of the prior term of its election to extend the Term hereof, or such option shall be deemed waived. If Tenant shall exercise such extension option(s), the parties will, at the request of either, execute an agreement in form for recording, evidencing such extension. If Tenant shall exercise such extension option(s), all references in this Lease to the Term hereof shall be deemed to mean the term as so extended, except where expressly otherwise provided.

Section 40.02. As of the first day of each extension term ("adjustment date"), the annual minimum rental shall be adjusted by multiplying the annual minimum rental payable hereunder during the last Lease Year of the prior Term, times a fraction having as its numerator the "Index" (hereinbelow defined) as of the adjustment date and as its denominator the Index in effect upon the commencement date of the initial term hereof; provided, however, the annual minimum rental payable subsequent to the adjustment date shall not be less than twenty-five (25%) percent greater than the annual minimum rental payable during the last preceding Lease Year of the prior Term.

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Section 40.03. The "Index" shall be defined as the United States Department of Labor, Bureau of Labor Statistics, "Consumer Price Index-All Urban Consumers - (CPI-U) N.Y., N.Y. -Northeastern N.J. (1982-84=100)". If publication of such Index shall be discontinued, the adjustments in the annual minimum rental provided for in this Lease shall thereafter be computed on the basis of such other official price index as shall be most nearly comparable thereto, and conversion tables, if any, issued upon the promulgation of such other official index, are to be used where applicable in making the computation hereunder.

IN WITNESS WHEREOF, the parties have hereunto set their hands and seals the day and year first above written.

ATTEST:

46.24 ASSOCIATES L.P.

By: **MIDDLESEX REALTY CORP.,**
General Partner

/s/ [ILLEGIBLE]

By: /s/ Robert C. Baker
Robert C. Baker

Title: PRESIDENT

(LANDLORD)

ATTEST:

PTC THERAPEUTICS, INC.

/s/ [ILLEGIBLE]
WITNESS

By: [ILLEGIBLE]

Title:

PRESIDENT

(TENANT)

STATE OF NEW YORK)
SS.:
COUNTY OF WESTCHESTER)

BE IT REMEMBERED, that on the 11th day of July, 2000, before me, the subscriber, a notary public of the State of New York, personally appeared Robert C. Baker, PRESIDENT of MIDDLESEX REALTY CORP., general partner of 46.24 ASSOCIATES L.P., who, I am satisfied, is the person who signed the within instrument; and I having first made known to him the contents thereof, he thereupon acknowledged that he signed, sealed with the corporate seal, and delivered the said instrument as such officer aforesaid, and that the within instrument is the voluntary act and deed of said corporation as said general partner, made by virtue of the authority of its board of directors.

/s/ Richard A. Kaufman
NOTARY PUBLIC

RICHARD A. KAUFMAN
Notary Public, State of New York
No. 4875196
Qualified in Westchester County
Commission Expires October 6, 2000

STATE OF NEW JERSEY)
SS.:
COUNTY OF MIDDLESEX)

BE IT REMEMBERED, that on the 12th day of Oct, 2010, before me the subscriber, a notary public of the State of New Jersey, personally appeared Stuart Peltz, of PTC THERAPEUTICS, INC., who, I am satisfied, is the person who signed the within instrument; and I having first made known to him the contents thereof, he thereupon acknowledged that he signed, sealed with the corporate seal, and delivered the said instrument as such officer aforesaid, and that the within instrument is the voluntary act and deed of said corporation, made by virtue of the authority of its board of directors.

/s/ Anne Armitage
NOTARY PUBLIC

Anne Armitage
Notary Public of New Jersey
No Commission Expires July 6, 2004

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EXHIBIT A
DEMISED PREMISES
Lot No. 24
Lot No. 25
Site Plan Approval
Tenant
By
Landlord
By
EXHIBIT A

EXHIBIT B

WORK LETTER TO BE ATTACHED TO
LEASE WITH PTC THERAPEUTICS, INC.
100 CORPORATE COURT
MIDDLESEX BUSINESS CENTER
SOUTH PLAINFIELD, NEW JERSEY

Landlord agrees to perform the Landlord's Work set forth in this Exhibit B. Any work not expressly specified herein and any work necessary to comply with codes attributable to Tenant's use shall be furnished and installed at the sole cost and expense of Tenant. Except as specifically set forth below, any existing construction within the Demised Premises shall be accepted by Tenant in "as is" condition in accordance with the provisions of Section 5.01 of this Lease. Landlord shall:

1. Provide a watertight roof and windows.

2. INTENTIONALLY OMITTED.

3. Replace stained or damaged ceiling tiles.

4. Repair exterior fence enclosure.

5. Provide a canopy over the side entrance.

6. Replace broken windows and glass doors.

7. Provide HVAC in good working order.

8. Relamp light fixtures as needed.

9. The blue or brown mesh remaining along the window line in the lab and in the cooler will be removed.

10. The Landlord will clean or replace all HVAC distribution supply or return duct (flexible pipes), VAV boxes and registers, diffusers or grills.

11. Landlord shall remove the asbestos linings present in the fume hoods located in the Demised Premises.

12. The existing acid neutralization tank will be put by Landlord into clean operating condition consistent with all applicable governmental regulations;

MISCELLANEOUS

A. All "Tenant Extras" furnished by Landlord as may be hereafter agreed to, shall be computed at Landlord's cost plus a twenty-one (21%) percent administrative fee.

B. Credits to Tenant based upon deletions and reductions below Building standard set forth above, shall be computed based upon Landlord's unit cost therefor, without factor of overhead and profit.

C. All prices, if any, set forth in this Work Letter are predicated upon quotations now in the hands of Landlord. Such quotations, by their terms, expire at various intervals and accordingly these prices are subject to variation based upon market conditions following the expiration of such quotations.

D. All prices are subject to inclusion of applicable taxes, but Landlord's overhead and profit

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shall be computed without regard to such taxes.

E. Landlord shall furnish Tenant with a statement(s) computing the net Tenant extras or credits due to Landlord or Tenant, as the case may be. Any amount due to Landlord shall be due and payable in full simultaneously with the delivery by Tenant to Landlord of the authorization for such work. Any credit due Tenant shall give rise to a reduction in the first installments of minimum rent and additional rent until such credit has been exhausted.

F. Prior to or promptly after execution of this Lease, Tenant shall furnish Landlord with design criteria and specifications necessary to enable Landlord to comply with its obligations above. All authorizations, deletions and implementations of the foregoing Work Letter shall be in writing and confirmed by authorized representatives of Landlord and Tenant.

G. If there shall be any conflict between the provisions of this Work Letter and the final approved Plans, the final approved Plans shall govern and control.

H. Landlord reserves the right to substitute for any materials and equipment specified herein, materials and equipment of substantially equal quality, provided Tenant's architect approves of the same, which approval shall not be unreasonably withheld or delayed.

I. Unless specifically stated in this Exhibit B or this Lease to the contrary, and notwithstanding anything contained on any plans or drawings, Tenant, and not Landlord, shall be responsible for furnishing and installing at its sole cost and expense any and all furniture, Tenant fixtures, appliances, shelving, cabinetry, phone systems, computer wiring and the like for the Demised Premises in accordance with the provisions of this Lease.

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EXHIBIT C

[B A N K]

[NAME ALL LANDLORDS]
c/o National Realty & Development Corp.
3 Manhattanville Road
Purchase, New York 10577

RE: Irrevocable Letter of Credit No.

Accountee: [Name of Tenant]

Gentlemen:

We hereby issue our Irrevocable Letter of Credit No. in favor of [Name All Landlords] at the request of [Name of Tenant] whose address is , for an aggregate amount of [Amount as required by Lease] and /l00 Dollars (U.S. $) effective immediately.

Funds are available against your signed draft(s) drawn on [Name of Bank] signed by an authorized representative of National Realty & Development Corp. mentioning this Letter of Credit No. accompanied by a written statement signed by an authorized representative of National Realty & Development Corp. requesting to draw upon a specified dollar amount on Letter of Credit No. as follows:

"This drawing in the amount of $ is pursuant to Lease dated , 2000 between [Name All Landlords], as Landlord, and [Name of Tenant], as Tenant."

This Letter of Credit shall expire on [Expiration Date of the Lease].

The beneficiaries named above shall be given not less than thirty (30) days written notice of the cancellation or non-renewal of the Letter of Credit. The beneficiaries of this Letter of Credit may draw upon the entire amount of the Letter of Credit if they shall receive notice of its cancellation or non-renewal or if it shall expire prior to the Expiration Date (as defined in the Lease) of the Lease. Partial drawings are permitted under this Letter of Credit.

[B A N K]

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EXHIBIT D

SUBORDINATION, NONDISTURBANCE AND ATTORNMENT AGREEMENT

by and among

PTC THERAPEUTICS, INC., as Tenant

and

46.24 ASSOCIATES, L.P., as Mortgagor

and

THE TRAVELERS LIFE AND ANNUITY COMPANY, as Mortgagee

Dated: June , 2000

Record and Return to:

Windels, Marx, Davies & Ives
120 Albany Street Plaza
New Brunswick, New Jersey 08901
Attn: Howard P. Lakind, Esq.

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SUBORDINATION, NONDISTURBANCE AND ATTORNMENT AGREEMENT

THIS SUBORDINATION, NONDISTURBANCE AND ATTORNMENT AGREEMENT, made this day of April, 2000 by and among

PTC THERAPEUTICS, INC., a Massachusetts corporation, having its principal office at 2 Chestnut Street, Grafton, Massachusetts 01519 (the "Tenant"),

46.24 ASSOCIATES, L.P., a Delaware limited partnership with its principal office located 3 Manhattanville Road, Purchase, New York 10577 (the "Mortgagor").

and

THE TRAVELERS LIFE AND ANNUITY COMPANY, having an office located at One Tower Square, 2 SHS, Hartford, Connecticut 06183 (the Mortgagee").

W I T N E S S E T H:

WHEREAS, the Tenant has entered into a certain Lease dated , 2000 (the "Lease"), with Mortgagor as landlord, covering all or a portion of those premises (the "Leasehold Premises") in the Borough of South Plainfield, County of Middlesex, State of New Jersey, all as more particularly described in SCHEDULE "A" attached hereto and made a part hereof (the "Mortgaged Premises"); and

WHEREAS, the Mortgagee has agreed to make or has made a mortgage loan in the aggregate principal amount of $4,609,851.06 to the Mortgagor (the "Loan"), which Loan is secured by a certain First Mortgage and Security Agreement dated May 16, 1984, as modified by certain Note and Mortgage Modification Agreement, dated as of February 14, 1995 but effective as of May 1, 1994 and modified by that certain Mortgage Modification Agreement dated as of April 30, 1999 granted by the Mortgagor to the Mortgagee upon the Mortgaged Premises (the "Mortgage"); and

WHEREAS, the execution and delivery of this Subordination, Nondisturbance and Attornment Agreement is a condition to the making of the Loan.

NOW, THEREFORE, in consideration of the sum of One Dollar ($1.00) by each party in hand paid to the other, receipt of which is hereby acknowledged, and in consideration of the premises and the mutual covenants and agreements hereinafter contained, the parties hereto, intending to be legally bound hereby, hereby agree as follows:

1. The Tenant hereby covenants and agrees:

(a) subject to this Agreement, the Lease and the Tenant's leasehold estate and any and all estates, options, liens and charges therein contained or created thereby are, and shall be and remain, subject and subordinate in all respects to the lien and effects of the Mortgage and to all of the terms, conditions, and provisions thereof, with the same force and effect as if the Mortgage had been executed, delivered and duly recorded prior to the execution and delivery of the Lease;

(b) from time to time, upon request by the Mortgagee, it shall forthwith provide the Mortgagee within ten (10) business days of such request with an estoppel certificate certifying that, to the best of its knowledge, no defaults, claims, offsets or events, or situations which, with the passage of time, could become a default or the basis for a

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claim or offset against the Mortgagor by the Tenant, exist under the Lease or, if the same exist, certifying and describing such items as are in existence;

(c) it will forward to the Mortgagee copies of any notice, claim or demand given or made by the Tenant to or on the Mortgagor, in all cases concurrently with forwarding same to the Mortgagor, such copies to be provided to the Mortgagee by the same method of sending or mailing as the statement, notice, claim or demand was made or given to or on the Mortgagor;

(d) without the prior written consent of the Mortgagee (i) no rent or other sums due under the Lease shall be paid more than thirty (30) days in advance of the due date therefor established by the Lease, except the security deposit, if any, (ii) no modifications shall be made in the provisions of the Lease nor shall the term be extended or renewed, except as provided therein, (iii) the Lease shall not be terminated except as provided therein and in this agreement nor shall the Tenant tender or accept a surrender of the Lease, (iv) it shall only sublet the Leasehold Premises or assign the Tenant's interest in the Lease in accordance with the provisions of said Lease, and (v) it shall not subordinate the Lease except to the Mortgage;

(e) in the event of any act or omission by the Mortgagor which would give the Tenant the right to terminate the Lease or to claim a partial or total eviction, reduce rents or to credit or offset any amounts against future rents, the Tenant will not exercise such right (i) until it shall have given written notice of such act or omission to the Mortgagee, and (ii) until a reasonable time for remedying such act or omission shall have elapsed following such giving of notice, and if it so elects, the Mortgagee shall have the right to cure any default by the Mortgagor under the Lease, including, if necessary to cure any such default, the right of access to the Leasehold Premises in accordance with the terms of the Lease;

(f) notices required to be given to the Mortgagee under this Agreement will be given to any successor-in-interest of the Mortgagee under the Mortgage provided that, prior to the event for which notice is required to be given to the Mortgagee, such successor-in-interest of the Mortgagee shall have given written notice to the Tenant of its acquisition of the Mortgagee's interest therein, and designated the address to which such notice is to be directed;

(g) if the holder of the Mortgage (as now or hereafter constituted), or anyone claiming from or through any such holder, shall enter into and lawfully become possessed of the Mortgaged Premises, or shall succeed to the rights of the Mortgagor under the Lease, either through foreclosure of said

Mortgage or otherwise howsoever, (i) the Tenant shall attorn to, and recognize, such holder or anyone claiming from or through such holder as its landlord under the Lease for the unexpired balance of the term of the Lease and any extension or renewal thereof, subject to all of the terms and conditions of the Lease provided such successor landlord recognizes Tenant's rights under the Lease, and (ii) the Tenant shall make all payments payable by the Tenant under the Lease directly to the holder of the Mortgage upon such holder's written instructions to the Tenant; and if, by operation of law, the institution of any action or other proceedings by the Mortgagee under the Mortgage or the entry into and taking, possession of the Mortgaged Premises shall result in the cancellation or termination of the Lease or the Tenant's obligations thereunder, the Tenant shall, upon request, execute and deliver a new lease of the Leasehold Premises pursuant to the Lease, containing the same terms and conditions as the Lease, except that the term and any extension thereof shall be the unexpired term and unexpired extended term or terms of the Lease as of the date of execution and delivery of said new lease;

(h) the Mortgagee shall have no responsibility, liability or obligation to cure any defaults by the Mortgagor under the Lease, nor be subject to claims, defenses or offsets under the Lease or against the Mortgagor possessed by the Tenant and which arose or

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existed prior to actual foreclosure of the Mortgage or recording of a deed in lieu of foreclosure or entry under and taking possession of the Mortgaged Premises by the Mortgagee except to the extent such claim, defense or offset is continuing as of the date of Mortgagee takes possession of the Mortgaged Premises. If the Mortgagee forecloses the Mortgage or takes title to the Mortgaged Premises pursuant to a deed in lieu of foreclosure or enters upon and takes actual possession of the Mortgaged Premises, the Mortgagee or any other purchaser at such foreclosure sale shall do so free and clear of all prior defaults, claims, defenses or offsets which the Tenant might have against any prior landlord (including the Mortgagor), and shall not be liable or responsible to the Tenant for any act or omission of any prior landlord (including the Mortgagor) except to the extent such act is continuing as of the date the Mortgagee succeeds to the interest of Landlord under the Lease, or be responsible or liable for any deposit or security which was delivered by the Tenant to any prior landlord (including the Mortgagor) but which was not subsequently delivered to the Mortgagee, or be bound by any rent or additional rent which the Tenant might have paid for more than the current month to any prior landlord (including the Mortgagor) but which was not subsequently delivered to the Mortgagee, or be obligated or liable to the Tenant with respect to the construction and completion of any improvements on the Mortgaged Premises for the Tenant's use, enjoyment or occupancy, or be bound by any restriction on competition beyond the Mortgaged Premises contained in the Lease, or be bound by any amendment or modification of the Lease made without the Mortgagee's prior written consent;

(i) the institution of any action or other proceedings by the Mortgagee under the Mortgage in order to realize upon the Mortgagor's interest in the Mortgaged Premises shall not, by operation of law or otherwise, result in the cancellation or termination of the Lease or the Tenant's obligations thereunder;

(j) any right of Tenant to make any claim or receive any proceeds arising out of taking by eminent domain shall be subject and subordinate to the rights of the Mortgagee; and

(k) Tenant hereby indemnifies and saves Mortgagee harmless against any claim, damage, liability, costs, penalties or fines which the Mortgagee may suffer as a result of air, land or water pollution caused by Tenant in its use or occupancy or manner of use or occupancy of the Mortgaged Premises or in its storage, handling, possession, transportation and or disposal of any hazardous or toxic substances, waste or materials (including, without limitation, PCB's or asbestos) within the Mortgaged Premises.

2. The Mortgagee hereby agrees:

(a) so long as the Tenant is not in default (beyond all applicable periods given the Tenant under the Lease to cure such default) and shall pay the rents and additional rents thereunder, and shall fully comply with and perform all the terms, covenants, conditions and provisions of the Lease on the part of the Tenant thereunder to be complied with and performed (i) the Tenant's possession and occupancy of the Leasehold Premises and the Tenant's rights and privileges under the Lease, or any extension or renewal thereof which may be effected in accordance with the terms of the Lease, shall not be disturbed by the Mortgagee or any successor-in-interest to the Mortgagee; (ii) the Mortgagee shall not join the Tenant as party to any action or proceeding brought as a result of a default under the Mortgage for the purposes of terminating the Tenant's interest and estate under the Lease, and subject further to the condition that the Mortgagee shall not be bound by any rent or other payment which the Tenant might have paid more than thirty (30) days in advance of the time stipulated for payment under the Lease or by any amendment or modification of the Lease made without its written consent; and

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(b) if the interest of the Mortgagor shall vest in the Mortgagee by reason of foreclosure or any other procedures brought by it, or in any other manner, the Mortgagee and its successors-in-interest shall have the option, but not the obligation to cure any defaults of the Mortgagor, and, upon such vesting, agrees to be bound by all of the undischarged obligations of Mortgagor under the Lease occurring after such foreclosure or other action.

3. The Tenant hereby represents and warrants that:

(1) the Lease is in full force and effect;

(2) neither the Tenant nor, to the best knowledge of the Tenant, the Mortgagor is in default in the performance of or compliance with any provision of the Lease;

(c) the Tenant has not received any notice of default or termination of the Lease;

(d) the Lease is a complete statement of the agreement of the parties thereto with respect to the leasing of the Leasehold Premises;

(e) the Tenant has no option under the Lease to purchase all or any portion of the Mortgaged Premises; and

(f) the Tenant is the sole owner of the leasehold estate created thereby.

4. The Tenant acknowledges that Mortgagor has executed or will execute an Absolute Assignment of Leases and Rents to the Mortgagee. The Mortgagor hereby irrevocably authorizes and directs the Tenant, upon receipt from the Mortgagee of written notice to do so, to pay all rents and other monies payable by the Tenant under the Lease to or at the direction of the Mortgagee. The Mortgagor irrevocably releases the Tenant from any liability to the Mortgagor for all payments so made, and the Mortgagor agrees to defend, indemnify and hold the Tenant harmless from and against any and all claims, demands, losses, or liabilities asserted by, through, or under the Mortgagor (except by the Mortgagee) for any and all payments so made. The Tenant agrees that upon receipt of such notice it will pay all monies then due and becoming due from the Tenant under the Lease to or at the direction of the Mortgagee notwithstanding any provision of the Lease to the contrary. Such payments shall continue until the Mortgagee directs the Tenant otherwise in writing. The Tenant agrees that neither the Mortgagee's demanding or receiving any such payments, nor the Mortgagee's exercising any other right, remedy, privilege, power or immunity granted by the Lease or this Agreement will operate to impose any liability upon the Mortgagee for the performance of any obligation of the Mortgagee under the Lease unless and until the Mortgagee elects otherwise in writing or unless the Mortgagee takes possession of the Mortgaged Premises and assumes the functions of a landlord.

5. Any notice, demand or consent hereunder shall be in writing any may be given, sent by a nationally recognized overnight courier service that provides a receipt for delivery, sent by facsimile, or mailed by registered or certified mail, return receipt requested, addressed, to the Mortgagee at the address set forth on the first page of this Agreement. Any party may designate a new address by notice in writing to the other party. Any notice given in accordance herewith shall be effective upon delivery, sending or deposit in the United States mails in accordance herewith.

6. This Agreement shall be binding upon and inure to the benefit of the successors and assigns of each of parties hereto. The term "Mortgagee" shall include the respective holders from time to time of the Mortgage (as now or hereafter constituted), the term "Mortgagor" shall be synonymous with the term "Landlord" during the term of the Mortgage and the terms "Landlord" and "Tenant" shall include the holder from time to time of the lessor's interest, and the holder from time to time of the lessee's interest, respectively, in the Lease.

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7. Any claim by the Tenant against the Mortgagee under the Lease or this Agreement shall be satisfied solely out of the interest of the Mortgagee in the Mortgaged Premises and the proceeds thereof and the Tenant shall not seek recovery against or out of any other assets of the Mortgagee.

8. This Agreement shall be governed by and construed under the laws of the State of New Jersey.

9. This Agreement may be executed in separate counterparts, all of which shall constitute a single instrument.

IN WITNESS WHEREOF, the Tenant, the Mortgagor and the Mortgagee have caused this Subordination, Nondisturbance and Attornment Agreement to be duly executed and delivered, all as of the day and year above written.

TENANT:

[ATTEST:] [WITNESS:]

PTC THERAPEUTICS, INC.

By: /s/ Michael Worm [ILLEGIBLE]
Name and Title Michael Worm [ILLEGIBLE] Witness

By: /s/ Stuart Peltz
Name and Title Stuart Peltz President

MORTGAGOR:

46.24 ASSOCIATES, L.P.

*** Attest/Witness***

By: _____
***Attestor/Witness Name

By: _____
Middlesex Realty Corp., President

MORTGAGEE:

THE TRAVELERS LIFE AND ANNUITY COMPANY

By: _____
Name: William P. Geary
Title:

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STATE OF :
:ss:
COUNTY OF :

BE IT REMEMBERED, that on this day of , 2000, before me, the subscriber, an officer duly authorized pursuant to N.J.S.A. 46:14-6 to take acknowledgments for use in the State of New Jersey, personally appeared , who I am satisfied is the person who executed the within Instrument as the of **PTC THERAPEUTICS, INC.,** the corporation named therein, and I having first known to **[him]** **[her]** the contents thereof, **[he]** **[she]** did thereupon acknowledge that the said Instrument made by the said corporation and delivered by **[him]** **[her]** as such officer, is the voluntary act and deed of said corporation, made by virtue of authority from its Board of Directors, for the uses and purposes therein expressed.

STATE OF :
:ss:
COUNTY OF :

BE IT REMEMBERED, that on this day of , 2000, before me, the subscriber, an officer duly authorized pursuant to law to take acknowledgments for use in the State of , personally appeared Robert C. Baker who, being duly sworn by me did depose and say he is the President of Middlesex Realty Corp., which corporation is the sole general partner of 46.24 Associates, L.P., who I am satisfied is the person who executed the within Modification Agreement as President of the sole general partner of the limited partnership named therein, and he did thereupon acknowledge that the said Modification Agreement made by the limited partnership and delivered by him as president of its sole general partner is the voluntary act and deed of said limited partnership made by virtue of authority under said limited partnership's partnership agreement, for the uses and purposes therein expressed.

Notary Public
of the State of New Jersey

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STATE OF :
:ss:
COUNTY OF :

BE IT REMEMBERED, that on this day of , 2000, before me, the subscriber, an officer duly authorized pursuant to law to take acknowledgements for use in the State of , personally appeared , who I am satisfied is the person who executed the within Modification Agreement as a Vice President of The Travelers Life And Annuity Company, the corporation named therein, and he did thereupon acknowledge that the said Modification Agreement made by the said corporation and delivered by him as such officer is the voluntary act and deed of said corporation, made by virtue of authority from its Board of Directors, for the uses and purposes therein expressed.

Notary Public
of the State of New Jersey

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SCHEDULE "A"

ATTACHED TO AND MADE A PART OF THAT CERTAIN
SUBORDINATION, NONDISTURBANCE AND ATTORNMENT
AGREEMENT, DATED AS OF , 2000

Description of the Mortgaged Premises

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EXHIBIT E

PTC TENANT FIT UP
LIST

6/21/2000 SCOPE OF WORK

DEMOLITION	CEILING TILE REMOVAL WALL REMOVAL OBSOLETE SYSTEMS AND WIRING REMOVAL DISPOSAL AND CLEAN UP
CONSTRUCTION	FLOOR PLAN CHANGES:5-7 OFFICES CEILING, REPLACEMENT NMR ROOM CONSTRUCTION INSTALL SHOWERS CONSTRUCT RECEPTION AREA ADDITIONAL EXECUTIVE OFFICE RENOVATE or REPLACE WINDOW TREATMENTS UPGRADE LUNCHROOM FACILITIES, DISHWASHER ETC
PAINTING	ENTIRE INTERIOR
CARPET	ENTIRE ADMINISTRATIVE AREA SELECTED OUTER OFFICES
PHONE SYSTEM	NEW SYSTEM AND WIRING
WIRING	COMPLETE NEW WIRING FOR VOICE/DATA SYSTEM CHECK, RENOVATE A-V SYSTEMS FOR LARGE MEETI ROOM
SECURITY	NEW KEY CARD ENTRY SYSTEM UPGRADE FIRE ALARM UPGRADE INTERNAL SECURITY SYSTEM
CASEWORK	RENOVATE/REPLACE 10 (MIN) ISLANDS OF CASE WORK AND T INSTALLATION OF WORKSPACE CARRELS FOR TECHNICAL ST 12/15
FUME HOODS	REPLACE EXISTING FUME HOODS WITH LARGER MODELS, - BENCH TOP WITH 2 FLAMMABLE CABINETS BELOW EACH INSTALL ADDITIONAL HOODS UPGRADE PLUMBING & WIRING FOR EACH HOOD UPGRADE EXHAUST SYSTEMS, DUCTS, FANS etc “MAKE-UP” AIR SYSTEM FOR HOODS INSTALLED (NEEDED TO BALANCE HVAC)
ARCHITECTURAL & ENGINEERING	BUILDING DRAWINGS FOR FLOOR PLAN AND “AS BUILT” MECHANICALS
WATER TREATMENT	RE-COMMISSION, UPGRADE DEIONIZED WATER SYSTEM REPLACE MILLIPORE WATER FILTRATION SYSTEM (OBSOLETE REPLACE MONITORING AND CHEMICAL DELIVERY SYSTEM FO ACID TANK

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FIRE PROTECTION	HORNS/STROBE SYSTEM BROUGHT TO CODE (SPLIT WITH NATIONAL) 15-20 NEW FIRE EXTINGUISHERS
BUILDING SYSTEMS	COMPRESSOR UPGRADE REPAIR GENERATOR (DOOR), START-UP INSPECTION, LOAD TESTING AND MAINTENANCE CONTRACT INSTALL VACUUM SYSTEM FOR LAB USE
ELECTRICAL	WIRING FOR NEW CONSTRUCTION SYSTEM TRACE FOR ALL CIRCUITS AND PANEL RE-LABELING SYSTEM UPGRADE FOR “UPS”
PLUMBING	SYSTEM TRACE AND INTEGRITY CHECK FOR LAB WATER SYST SHOWER INSTALLATION SAFETY STATION (SHOWERS, EYEWASH) UPGRADE SYSTEM TRACE AND INTEGRITY CHECK FOR SPECIALIZED GA
GLASSWARE CLEANING	REPLACE STEAM GENERATOR REPLACE STERILIZER (RECONDITIONED UNIT) REPLACE WASHER
COLD ROOM	SYSTEM CHECK AND RE-COMMISSION, INCLUDING CONTROL SYSTEMS

FIRST MODIFICATION and EXTENSION OF LEASE

This FIRST MODIFICATION and EXTENSION OF LEASE (this "Modification") is made as of the 1st day of December, 2003 (the "Building 200 Premises Commencement Date") by and between **46.24 ASSOCIATES L.P.,** a Delaware limited partnership, for the purposes of this Modification, having its office and P.O. Address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord"), and **PTC THERAPEUTICS, INC,** a Delaware corporation, having an office 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (hereinafter referred to as "Tenant").

W I T N E S S E T H:

WHEREAS, Landlord, and Tenant entered into that certain lease (herein referred to as the "Lease") dated as of July 11, 2000, as amended by letter agreements dated July 10, 2000 and December 14, 2000, by which Tenant leased from Landlord, and Landlord leased to Tenant, certain premises in building #100 (the "Building") of the Middlesex Business Center (the "Center") in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX, and STATE OF NEW JERSEY, which premises consist of approximately 21,700 square feet and are more particularly described in the Lease (such premises are herein referred to as the "Existing Premises"); and

WHEREAS, Tenant is currently occupying certain space (the "Additional Premises"), also located in building #100 of the Center, which premises are directly adjacent to the Existing Premises, which Tenant initially occupied pursuant to a Sub-Tenancy Agreement dated June 1, 2001, between Catalyst Communications, Inc. ("Catalyst") as sublessor, and Tenant as Sublessee. (The Existing Premises, together with the Additional Premises, constitute the total leasable area of the Building, 30,000 square feet,)

WHEREAS, Landlord and Tenant now desire to modify the Lease in certain respects;

NOW, THEREFORE, for TEN and 00/100 ($10.00) DOLLARS and other good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, the parties hereto agree as follows:

1. Tenant hereby leases from Landlord, and Landlord hereby leases to Tenant, for a term commencing on January 1, 2003 (the "Effective Date") and ending on the Revised Expiration Date (as hereinafter defined) (or that date on which the Lease shall be sooner terminated) approximately 8,300 square feet of floor space in the Building more particularly indicated and described by hatching on the left (\\\) on the plot plan annexed hereto as "Exhibit A" and hereby made a part hereof (such 8,300 square feet of floor space is hereinafter referred to as the "Additional Premises"). From and after the Effective Date, the Additional Premises shall be deemed to be part of the Demised Premises, as if the same was included within the Demised Premises as the same is defined by the Lease (so that from and after the Effective Date the Demised Premises shall consist of approximately 30,000 square feet). From and after the Effective Date, the plot plan attached to the Lease as Exhibit A thereto shall be deemed deleted from the Lease and the plot plan attached as Exhibit A to this Modification shall be deemed substituted therefore.

2. Tenant has examined the Additional Premises and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the Additional Premises, except as is in this Modification specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to its knowledge, without independent investigation, that except as disclosed in the Phase I Environmental Assessment prepared by Environmental Management Services dated June 19th, 2000, there were no environmental hazards present within the Additional Premises prior to Tenant's occupancy thereof. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation, examination, and inspection of the Additional Premises and Tenant accepts the Additional Premises "as is",

3. Tenant hereby leases from Landlord, and Landlord hereby leases to Tenant, for a term commencing on December 1, 2003 and ending on the Revised Expiration Date (or that date on which the Lease shall be sooner terminated) approximately 11,500 square feet of floor space in the building known as 200 Corporate Court in the Center, which floor space is more particularly indicated and described by cross hatching on the plot plan annexed hereto as "Exhibit A" and hereby made a part hereof (such 11,500 square feet of floor space is hereinafter referred to as the "Building 200 Premises"). From and after December 1, 2003 the Building 200 Premises shall be deemed to be part of the Demised Premises, as if the same was included within the Demised Premises as the same is defined by the Lease (so that from and after the Building 200 Premises Commencement Date the Demised Premises shall consist of approximately 41,500 square feet).

4.A. Tenant has examined the Building 200 Premises and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the Building 200 Premises, except as is in this Modification specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to its knowledge, without independent investigation, except as disclosed in the Phase I Environmental Assessment prepared by Environmental Management Services dated June 19th, 2000, there were no environmental hazards present within the Building 200 Premises prior to Tenant's occupancy thereof. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation, examination, and inspection of the Building 200 Premises and Tenant accepts the Building 200 Premises "as is" except as otherwise set forth herein.

B. Tenant, at Tenant's expense, shall pursuant to plans and specifications prepared by Tenant and approved by Landlord and otherwise subject to provisions of the law, including without limitation the provisions of the Lease: (i) perform such work as is needed so that the Building 200 Premises are demised separately from the remainder of the building of which such premises are a part, (ii) install demising walls with respect to the Building 200 Premises in the locations shown on the plan attached hereto as Exhibit A-l which is hereby made a part hereof, (iii) paint such demising walls a color of Tenant's choosing from Landlord's standard paint selections, and (iv) separate the utilities currently servicing the Building 200 Premises so that such premises are separately metered and that such meters measure only the consumption for the Building 200 Premises.

C. Tenant shall be responsible for performing such work as is needed so that the interior portion of the Additional Premises and Building 200 Premises are compliant with the ADA. Landlord represents that it has performed such work as is needed so that the exterior portion of the Additional Premises and the exterior of the Building 200 Premises are compliant with the Americans with Disabilities Act (the "ADA").

5. Landlord and Tenant agree that the Term of the Lease shall expire, notwithstanding anything to the contrary in Section 1,03 of the Lease, on July 31, 2009 (the "Revised Expiration Date").

6. Effective as of the Effective Date, the annual minimum rental payable under Section 3.01 of the Lease shall be as follows, notwithstanding anything in Section 3.01 of the Lease to the contrary,

(A) From the Rent Commencement Date (as defined in the Lease) through December 31, 2002 (inclusive): **ONE HUNDRED EIGHTY FOUR THOUSAND FOUR HUNDRED FIFTY AND 00/100 ($184,450.00) DOLLARS per annum – FIFTEEN THOUSAND THREE HUNDRED SEVENTY AND 83/100 ($15,370.83) DOLLARS per month;** and

(B) From and including January 1, 2003 until March 31, 2004: **TWO HUNDRED FIFTY SEVEN THOUSAND SEVEN HUNDRED THIRTY AND 04/100 ($257,730.04) DOLLARS per annum – TWENTY ONE THOUSAND FOUR HUNDRED SEVENTY SEVEN AND 50/100 ($21,477.50) DOLLARS per month.**

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(C) From April 1, 2004 (the "Building 200 Rent Commencement Date") through July 31, 2009: **THREE HUNDRED SEVENTY THREE THOUSAND FIVE HUNDRED AND 00/100 ($373,500.00) DOLLARS per annum - THIRTY ONE THOUSAND ONE HUNDRED TWENTY FIVE AND 50/100 ($31,125.00) DOLLARS per month;**

7. Provided that (i) Tenant is in compliance with the provisions of that certain License Agreement dated September 22, 2003 by and between Landlord and Tenant (the "License Agreement") with respect to Renovations Work, and (ii) Tenant has complied with the provisions of Section 5.05(c) of the Lease as same apply to the Renovations Work pursuant to said License Agreement and (iii) Tenant has completed all work necessary to renovate, fixture and equip the Additional Premises and Building 200 Premises for use in accordance with the provisions of Article 2 of the Lease (the "Additional Tenant Improvements"), and (iv) Tenant complies with the requirements of Section 5.05(C) of the Lease, and provided further that the Lease shall not be terminated prior to the dates Tenant is entitled to such credits, Tenant shall be entitled to a credit of $31,125.00 against the payment of the monthly installments of annual minimum rent due under the Lease for the months of July 2004 and August 2004, and a credit of $31,125.00 against the payment of annual minimum rent due under the Lease for the months of June 2009 and July 2009 (and such credits shall be in lieu of the rent abatement set forth in that certain letter agreement dated December 14, 2000 between Landlord and Tenant.)

8. Section 5.05(B) of the Lease is hereby amended in its entirety to read as follows:

"Subject to completion of the Additional Tenant Improvements in accordance with Tenant's plans and specifications which shall be approved by Landlord in advance, and the provisions set forth in Section 5.05(C) of the Lease, Landlord shall reimburse Tenant for amounts actually expended by Tenant for a portion of the construction of the Additional Tenant Improvements, in an amount not to exceed the sum of $159,700.00, and for partitioning and installation of separate HVAC systems (the "HVAC Improvements") in an amount not to exceed $6,000 (both amounts together, the "Tenant Allowance"). Such reimbursement shall be in the form of a credit to be taken by Tenant against the first payments of annual minimum rental payable hereunder which accrue subsequent to the completion of the Additional Tenant Improvements and the HVAC Improvements, which shall be credited starting no earlier than August 31, 2004 until exhausted.

The portion of the Additional Tenant Improvements for which the Tenant Allowance shall be paid shall be only that portion thereof that is office installation work (e.g. partitioning, doors, electrical, etc., including, without limitation, upgrading and replacing of existing mechanical systems in the Demised Premises) and in no event shall the Tenant Allowance be used for any work related to Tenant's trade fixtures or equipment (such portion of the Additional Tenant Improvements for which Tenant may receive the Tenant Allowance is hereinafter called the "Tenant Allowance Work"). It is acknowledged and agreed by Tenant that in the event that the monies expended by Tenant for the completion of the Tenant Allowance Work and the HVAC Improvements are less than the amount of the Tenant Allowance, Tenant shall not be entitled to any credit for the unused portion thereof, nor is Tenant entitled to any additional sums from Landlord in the event that the monies expended by Tenant in connection with the completion of the Tenant Allowance Work, the HVAC Improvements or the Additional Tenant Improvements exceeds the amount of the Tenant Allowance."

9. Tenant and Landlord acknowledge that Tenant has held annual minimum rent and additional rent accruing with respect to the Additional Premises for the period of time from and after January 1, 2003 in escrow pending resolution of the status of the Catalyst Lease and the Catalyst-Sublease (as defined herein). Upon execution of this Modification by Tenant, Tenant shall pay to Landlord annual minimum rent and additional rent for the Additional Premises for the period from January 1, 2003 through the last day of January 2004 in the amount of **ONE HUNDRED SIXTEEN THOUSAND NINETY FIVE AND 57/100 ($116,095.57) DOLLARS** (which together with rent previously paid by Tenant with respect to the Existing Premises, the Landlord accepts in full satisfaction of the amount owed pursuant to Section 6(B), above, for the period from January 1, 2003 through February 29 , 2004, subject to year end reconciliation for additional rent.). As between Landlord and Tenant, Landlord hereby waives any rights or remedies it may have (either under the Lease, the Catalyst Lease, or the Catalyst Sublease) with

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respect to such payments, the underlying rent or costs to which the payments relate, or the timing of such payments.

10. Landlord represents and warrants, and Tenant acknowledges, that the lease by and between Landlord and Catalyst Communications, Inc. ("Catalyst") by which Catalyst leased the Additional Premises (the "Catalyst Lease") was terminated as to the Additional Premises effective as of December 31, 2002 and that, accordingly, that certain sublease by and between Catalyst and Tenant by which Tenant subleased the Additional Premises from Catalyst also

terminated as of December 31, 2002 (the "Catalyst Sublease"). In recognition of Tenant's payments hereunder, Landlord hereby indemnifies and saves harmless Tenant from and against any claims and all loss, cost, liability, damage and/or expense, including but not limited to reasonable counsel's fees, penalties and fines, incurred in connection with or arising from the payment of annual minimum rent or additional rent from January 1, 2003 forward under the Catalyst Lease or the Catalyst Sublease, Tenant's good faith efforts to preserve rents on the Additional Premises in an escrow account pending resolution of issues between Landlord and Catalyst, or any other financial issues regarding the payment of rent or additional rent to Landlord by Catalyst or to Catalyst by Tenant,

11. Article 40 of the Lease is hereby deleted and replaced with the following quoted language:

"ARTICLE 40. OPTIONS FOR EXTENSION AND ADDITIONAL SPACE

Section 40.01. Tenant shall have the option, provided it is not in default hereunder, to extend the term of this Lease for ONE (1) successive additional term of FIVE (5) years, from August 1, 2009 through July 31, 2014, upon the same terms and conditions as provided herein, except that the fixed annual rent during said extension period shall be as provided below, and except that Tenant shall have no further extension options. Tenant shall give written notice to Landlord prior to November 1, 2008 of its election to extend the term hereof, or such option shall be deemed waived. If Tenant shall exercise such extension option, the parties will, at the request of either, execute an agreement in form for recording, evidencing such extension. If Tenant shall exercise such extension option, all references in this Lease to the term hereof shall be deemed to mean the term as so extended, except where expressly otherwise provided.

Section 40.02. If Tenant shall duly elect to extend the term of this Lease as herein provided, the fixed annual rent payable by Tenant during said extension term shall be **FOUR HUNDRED TWENTY NINE THOUSAND FIVE HUNDRED TWENTY FIVE AND 00/100** ($429,525.00) DOLLARS per annum- **THIRTY FIVE THOUSAND SEVEN HUNDRED NINETY THREE AND 001/000** ($35,793.75) DOLLARS per month.

Section 40.03. If at any time Landlord proposes to lease space within the building known as 200 Corporate Court in the Center, or enters into discussions with a potential tenant(s) for some or all of such space, Landlord shall give Tenant notice thereof (the "Start Notice") in order to provide Tenant with a reasonable right of first negotiation with respect to such additional space upon substantially similar terms and conditions as provided herein, except that the fixed annual rent applicable to such space shall be as negotiated in good faith by the parties. In the event that Landlord and Tenant do not enter into a lease for such space within thirty (30) days of the Start Notice, then Tenant shall, at its expense, within thirty (30) days of Landlord's request, undertake all actions necessary so that the utilities serving the Building 200 Premises are located solely within the Building 200 Premises, and not-otherwise in Building 200, and shall also take all actions necessary so that no utilities serving the portion of Building 200 not within the Building 200 Premises are located within the Building 200 Premises, such that the Building 200 Premises and the other portions of Building 200 can be used by two (2) (or more) unrelated tenants without any utilities located other than within the particular tenant's demised premises."

12. Tenant covenants, warrants and represents that it has dealt with no broker other than Colliers Houston & Co. respecting this Modification and that no conversations, correspondence or negotiations were had with any broker (except with Colliers Houston & Co.

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concerning the negotiation of the Lease) concerning the renting or leasing of the Additional Premises or Building 200 Premises. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations with any broker except said Colliers Houston & Co. regarding the Lease. Landlord shall pay any commissions owing to said Colliers Houston & Co. with respect to this Modification in accordance with separate agreement.

13. Upon execution of this Modification by Tenant, Tenant shall deliver to Landlord adequate and proper insurance policies with respect to the Additional Premises and the Building 200 Premises including, without limitation, comprehensive general liability insurance in the amounts specified in and in accordance with the provisions of Article 7 of this Lease.

14. Section 24.01 of the Lease is hereby deleted and replaced with the following quoted language:

"Tenant represents that its officer(s) executing tills lease have been duly authorized to do so by formal action of a duly-elected Board of Directors. Until such time as Tenant is a corporation whose shares are regularly and publicly traded on a recognized stock exchange, Tenant shall upon written request provide to Landlord a capitalization table showing ownership (by class and entity) of Tenant's capital stock prepared or reviewed by a certified public accountant."

Landlord hereby waives any right of termination of the Lease that may have accrued to Landlord pursuant to the former language of Section 24.01 of the Lease.

15. Except as expressly modified herein, all of the terms and conditions of the Lease shall continue unmodified and in full force and effect. Capitalized terms used herein without definition shall have the meaning given to such terms in the Lease. Obligations under this Agreement shall be deemed obligations under the Lease and that a default hereunder shall constitute a default under the Lease.

IN WITNESS WHEREOF, the parties have hereunto set their hands and seals as of the day and year first above written.

WITNESS:

/s/ [ILLEGIBLE]

46.24 ASSOCIATES L.P., a Delaware limited partnership

By: Middlesex Realty Corp., general partner

By: /s/ Robert C. Baker
Name: Robert C. Baker
Title: President

(LANDLORD)

PTC THERAPEUTICS, INC., a Delaware Corporation

By: /s/ Mark E. Boulding
Name: Mark E. Boulding
Title: Secretary of the [ILLEGIBLE]
(TENANT)

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L [ILLEGIBLE]

State of New York
}SS:
County of Westchester

On the 17th day of February in the year 2004 before me, the undersigned, a Notary Public in and for said State, personally appeared Robert C. Baker, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as President of MIDDLESEX REALTY CORP., general partner of 46.24 ASSOCIATES, INC., and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ WAYNE E. HELLER
Notary Public
WAYNE E. HELLER
Notary Public, State Of New York
No. 02HE6062858
Qualified In Westchester County
Commission Expires August 20, 2005

STATE OF NEW JERSEY)
} SS.:
COUNTY OF MIDDLESEX)

On the 9 day of Feb in the year 2004 before me, the undersigned, a Notary Public in and for said State, personally appeared Mark E. Boulding, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as Secretary of PTC THERAPEUTICS, and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument..

/s/ ANTHONY F. LEONETTI
Notary Public
ANTHONY F. LEONETTI
Notary Public, New Jersy
My Commission Expires November 29, 2005
Mail Boxes Etc. #912

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L [ILLEGIBLE]

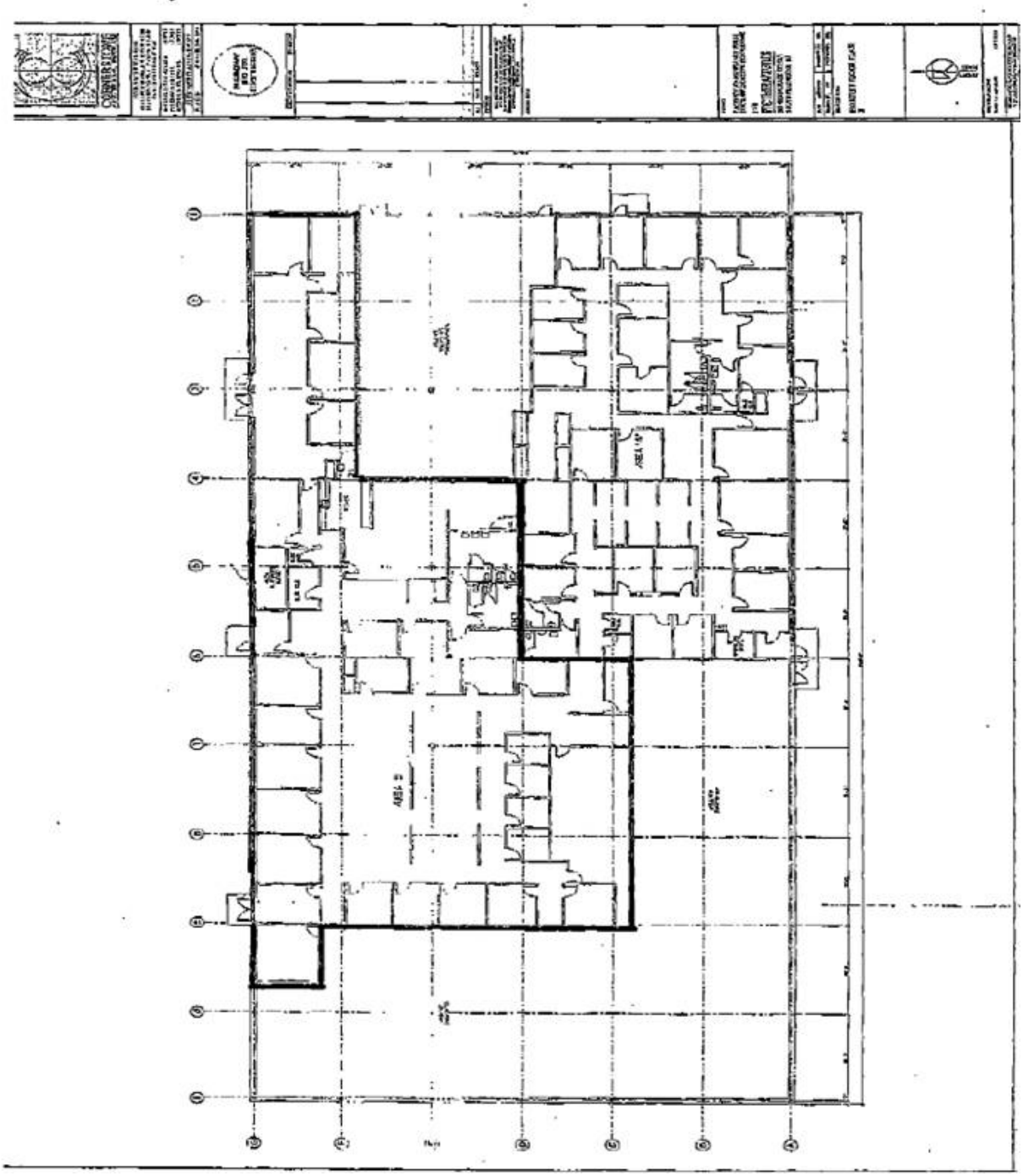
EXHIBIT A

Tenant
By
Landlord
By

EXHIBIT A

Exhibit H-1

A-1



SECOND MODIFICATION OF LEASE

This SECOND MODIFICATION OF LEASE (this "Modification") is made as of the 20th day of October, 2006 by and between **46.24 ASSOCIATES L.P.,** a Delaware limited partnership, having its office and P.O. Address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord"), and **PTC THERAPEUTICS, INC.** a Delaware corporation, having an office at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (hereinafter referred to as "Tenant").

W I T N E S S E T H:

WHEREAS, Landlord and Tenant entered into that certain lease (herein referred to as the "Lease") dated as of July 11, 2000, as amended by letter agreements dated July 10, 2000 and December 14, 2000, by which Tenant leased from Landlord, and Landlord leased to Tenant, certain premises in building #100 of the Middlesex Business Center (the "Center") in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX, and STATE OF NEW JERSEY, which premises consist of approximately 21,700 square feet and are more particularly described in the Lease; and

WHEREAS, Landlord and Tenant entered into that certain First Modification and Extension of Lease Agreement dated December 1, 2003 whereby the Demised Premises were increased by 8,300 square feet in building #100 (bringing the total leased space in building #100 to 30,000 square feet), as well as adding 11,500 square feet of space located in Building #200, such that the Demised Premises now contain 41,500 square feet.

WHEREAS, Landlord and Tenant now desire to modify the Lease in certain respects;

NOW, THEREFORE, for TEN and 00/100 ($10.00) DOLLARS and other good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, the parties hereto agree as follows:

1. Tenant hereby leases from Landlord, and Landlord hereby leases to Tenant, for a term commencing on January 1, 2007 (the "Effective Date") and ending on February 29, 2012 (the "Expiration Date") approximately 18,500 additional square feet of floor space in Building #200 more particularly indicated and described by hatching on the left (\\\) on the plot plan annexed hereto as "Exhibit A" and hereby made a part hereof (such 18,500 square feet of floor space is hereinafter referred to as the "Building #200 Additional Premises" and represents the balance of Building 200 not currently leased to Tenant so that following the Effective Date, the Lease shall demise all of Building #100 and all of Building #200 to Tenant). From and after the Effective Date, the Additional Premises shall be deemed to be part of the Demised Premises, as if the same was included within the Demised Premises as the same is defined by the Lease (so that from and after the Effective Date the Demised Premises shall consist of approximately 60,000 square feet). From and after the Effective Date, the plot plan attached to the Lease as Exhibit A thereto shall be deemed deleted from the Lease and the plot plan attached as Exhibit A to this Modification shall be deemed substituted therefore.

2. Tenant has examined the Building #200 Additional Premises and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the Building #200 Additional Premises, except as is in this Modification specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to its knowledge, without independent investigation, that except as disclosed in the Phase I Environmental Assessment prepared by Environmental Management Services dated June 19th, 2000, there are no environmental hazards present within the Building #200 Additional Premises. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation, examination, and inspection of the Building #200 Additional Premises and Tenant accepts the Building #200 Additional Premises "as is".

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3. Tenant shall be responsible for performing such work as is needed so that the interior portions of the Building 200 Additional Premises are compliant with the ADA. Landlord represents that it has performed such work as is needed so that the exterior portion of the Building 200 Additional Premises are compliant with the Americans with Disabilities Act (the "ADA").

4. Beginning on the Effective Date, the annual minimum rental payable under Section 3.01 of the Lease, as amended by Section 6 of the First Modification of Lease and this Modification, shall be as follows, notwithstanding anything in Section 3.01 of the Lease to the contrary:

From January 1, 2007 through December 31, 2009: **FIVE HUNDRED SEVENTY THOUSAND AND 00/100 ($570,000.00) DOLLARS per annum — FORTY SEVEN THOUSAND FIVE HUNDRED AND 00/100 ($47,500.00) DOLLARS per month; and**

From January 1, 2010 through February 28, 2012: **SIX HUNDRED FIFTEEN THOUSAND AND 00/100 ($615,000.00) DOLLARS per annum — FIFTY ONE THOUSAND TWO HUNDRED FIFTY AND 00/100 ($51,250.00) DOLLARS per month.**

Provided that Tenant is not then in default, and has not been in default, annual minimum rental shall abate during the months of January, 2007 and March, 2007.

5. Effective immediately, Article 40 of the Lease (as modified by the First Modification and Extension of Lease) is hereby deleted and replaced with the following quoted language in its entirety.

"ARTICLE 40. OPTION FOR EXTENSION

Section 40.01. Tenant shall have the option, provided it is not in default hereunder, to extend the term of this Lease for ONE (1) successive additional term of FIVE (5) years, from March 1, 2012 through February 28, 2017, upon the same terms and conditions as provided herein, except that the annual minimum rent during said extension period shall be adjusted as provided below, and except that Tenant shall have no further extension options. Tenant shall give written notice to Landlord prior to June 1, 2011 of its election to extend the term hereof, or such option shall be deemed waived. If Tenant shall exercise such extension option, the parties will, at the request of either, execute an agreement in form for recording, evidencing such extension. If Tenant shall exercise such extension option, all references in this Lease to the term hereof shall be deemed to mean the term as so extended, except where expressly otherwise provided.

Section 40.02. If Tenant shall duly elect to extend the Term as herein provided, the annual minimum rental payable by Tenant during said extension term shall be **SIX HUNDRED SEVENTY SIX THOUSAND EIGHT HUNDRED AND 00/100 ($676,800.00) DOLLARS** per annum — **FIFTY SIX THOUSAND FOUR HUNDRED AND 00/100 ($56,400.00) DOLLARS** per month.

6. Effective immediately the December 14, 2000 Letter Agreement is hereby deleted in its entirety.

7. If Tenant elects to improve any of the space in Building #100 or the Building #200 Additional Premises prior to March 31, 2008, in accordance with Tenant's plans and specifications which shall be approved by Landlord in advance pursuant to the terms of the Lease (such approval not to be unreasonably withheld, conditioned or delayed), and provided that Tenant shall (i) provide Landlord with proof of payment (in the form of copies of paid invoices) to all contractors, laborers, and suppliers involved with the Improvements, (ii) provide Landlord with lien waivers from all contractors, laborers and suppliers involved with the Improvements, and (iii) provide Landlord with a copy of the Certificate of Occupancy or applicable building inspector sign-offs covering the improved portion of the Demised Premises upon completion of the Improvements, along with "as built" plans, then, in such event Landlord shall provide Tenant with a rent credit for amounts expended by Tenant for the construction of the Improvements, in an amount not to exceed the sum of SIX HUNDRED THOUSAND

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($600,000.00) Dollars (the "Allowance"). Provided that Tenant is not in default of the terms of this Lease, such reimbursement shall be in the form of a credit to be taken by Tenant against the payment of annual minimum rental due and payable (immediately following Tenant's prior compliance with the requirements of this paragraph) which shall be credited in alternating months against 100% of such alternating monthly payments until exhausted. The Allowance shall only be applied

towards approved Improvements in the Buildings or the Demised Premises, and shall not be applied towards Tenant's equipment, fixtures, inventory or other personal property charges.

Tenant shall not be entitled to any additional sums from Landlord in the event that the monies expended by Tenant in connection with the completion of the Improvements exceeds the amount of the Allowance.

Upon and after execution of this Modification by Tenant, Tenant shall have full and immediate access to the Building #200 Additional Premises to plan and commence architectural, engineering and construction activities.

8. At any time and from time to time during the Lease term (including any extension), if any space within the building in the Center known as 250 Corporate Court ("Building #250") is not then leased by Landlord to a third party, Tenant shall have the option (upon written notice to Landlord) to lease any Unit (as defined below) of such space, on the same terms and conditions as those provided herein, except that the fixed monthly rent applicable to such space during each month of its occupancy shall be equal to the number of square feet to be leased times the average monthly rent per square foot payable on the existing spaced leased under this Lease during such month, under Section 3.01 of the Lease as amended hereby. Tenant may only elect to lease a complete "Unit" or "Units" within Building #250. An exhibit showing the current approximate dimension and size of the Units is attached hereto as "Exhibit B". Tenant acknowledges that the Units depicted on Exhibit B may be changed from time to time by Landlord and that Tenant may only elect to lease a Unit as it exists at the time of its election.

9. If at any time Landlord proposes to lease (including any lease renewal or extension) space within Building #250, or enters into discussions with a potential tenant(s) for some or all of such space, Landlord shall provide Tenant with a reasonable right of first negotiation with respect to such additional space upon substantially similar terms and conditions as provided to such potential tenant. In the event that Tenant does not indicate in writing within five (5) business days that it intends to enter into a Lease with Landlord on the same terms as proposed by the potential tenant, and Landlord and Tenant do not enter into a lease for such space at 250 Corporate Court within thirty (30) days of Landlord's offer to Tenant, upon the same terms and conditions as offered by said potential tenant, then this right of first negotiation shall be null and void and of no further force and effect with respect to any subsequent rental to such tenant on the terms originally offered by said potential tenant. For avoidance of doubt, such reasonable right of first negotiation shall continue and/or be reinstated, as applicable, for any such space not leased to such tenant on the terms originally offered by said potential tenant.

10. If Tenant leases any space in Building #250 pursuant to Section 8 or 9 of this Modification, then Tenant may at any time elect to improve any of such leased space in Building #250, in accordance with Tenant's plans and specifications which shall be approved by Landlord in advance pursuant to the terms of the Lease (such approval not to be unreasonably withheld, conditioned or delayed), in which case Tenant shall (i) provide Landlord with proof of payment (in the form of copies of paid invoices) to all contractors, laborers, and suppliers involved with the Improvements, (ii) provide Landlord with lien waivers from all contractors, laborers and suppliers involved with the Improvements, and (iii) provide Landlord with a copy of the Certificate of Occupancy or applicable building inspector sign-offs covering the improved portion of the Demised Premises upon completion of the Improvements, along with "as built" plans. In such event, Landlord shall provide Tenant with a rent credit for amounts expended by Tenant for the construction of the Improvements in Building #250, in an amount not to exceed the sum of TWELVE ($12) Dollars per square foot of space so improved (the "Building #250 Allowance"), provided that, the Building #250 Allowance shall be adjusted downward by an amount equal to 20% for each full (but not partial) twelve-month period which has lapsed since the Effective Date. Provided that Tenant is not in default of the terms of this Lease, such reimbursement shall be in the form of a credit to be taken by Tenant against the payment of annual minimum rental due and payable (immediately following Tenant's prior compliance with

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the requirements of this paragraph) which shall be credited in alternating months against 100% of such alternating monthly payments until exhausted. The Building #250 Allowance shall only be applied towards approved Improvements in Building #250, and shall not be applied towards Tenant's equipment, fixtures, inventory or other personal property charges.

Tenant shall not be entitled to any additional sums from Landlord in the event that the monies expended by Tenant in connection with the completion of the Improvements in Building #250 exceed the amount of the Building #250 Allowance.

Upon and after the lease by Tenant of any space in Building #250, Tenant shall have full and immediate access to the leased Building #250 premises to plan and commence architectural, engineering and construction activities.

11. Subject to the requirements of local codes and all required municipal approvals, Tenant shall be permitted to construct a connecting building and/or corridor, of no more than 5,000 square feet, to connect 100 Corporate Court and 200 Corporate Court, and shall thereafter be permitted to occupy such connecting building and/or corridor rent free for the life of Tenant's occupancy of space in Building 100 and Building 200. Prior to having any communication with the local authorities regarding such proposed construction, Tenant shall first submit plans to Landlord for review, approval of which shall not be unreasonably withheld, conditioned or delayed, provided, however, that such consent shall be subject to the review and approval of any lender then in existence with respect to either building or otherwise encumbering the interest of Landlord or its affiliates in the Center and having any authority to approve or reject such a proposal. Any and all costs associated with such application and construction shall be paid by Tenant. Tenant shall be responsible for any increased taxes (of any type) associated with such construction assessed with regard to periods on or before the Expiration Date (but not any additional income taxes or other taxes payable by Landlord on account of Tenant's payment of such taxes on Landlord's behalf). Any approved structure shall be removed by Tenant at the expiration or earlier termination of the Term, at Landlord's option, and the area in which the structure is constructed shall be returned to the condition it was in prior to said construction. Tenant shall be solely responsible for any maintenance or other costs incurred by Landlord as a result of Tenant's installation described above during the Term and any Extension Term.

12. Tenant covenants, warrants and represents that it has dealt with no broker other than Colliers Houston & Co. respecting this Modification and that no conversations, correspondence or negotiations were had with any broker (except with Colliers Houston & Co. concerning the negotiation of the Modification) concerning the renting or leasing of the Building 200 Additional Premises. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations with any broker except said Colliers Houston & Co. regarding the Lease. Landlord shall pay any commissions owing to said Colliers Houston & Co. with respect to this Modification in accordance with separate agreement.

13. Within seven (7) days after Tenant's execution of this Modification, Tenant shall provide Landlord with the sum of $53,890.00 so that the Security held by Landlord pursuant to Article 39 of the Lease shall be $100,000.00.

14. Upon execution of this Modification by Tenant, Tenant shall deliver to Landlord adequate and proper insurance policies with respect to the Building #200 Additional Premises including, without limitation, comprehensive general liability insurance in the amounts specified in Article 7 of this Lease.

15. Except as expressly modified herein, all of the terms and conditions of the Lease shall continue unmodified and in full force and effect. Capitalized terms used herein without definition shall have the meaning given to such terms in the Lease. Obligations under this Agreement shall be deemed obligations under the Lease and that a default hereunder shall constitute a default under the Lease.

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IN WITNESS WHEREOF, the parties have hereunto set their hands and seals as of the day and year first above written.

WITNESS:

[ILLEGIBLE]

46.24 ASSOCIATES L.P., a Delaware limited partnership

By: Middlesex Realty Corp., general partner

By: /s/ Robert C. Baker
Name: Robert C. Baker
Title: President

(LANDLORD)

[ILLEGIBLE]
[ILLEGIBLE]

PTC THERAPEUTICS, INC., a Delaware corporation

By: /s/ [ILLEGIBLE]
Name: [ILLEGIBLE]
Title: [ILLEGIBLE]

(TENANT)

State of New York
} SS:
County of Westchester

On the 19th day of Oct. in the year 2006 before me, the undersigned, a Notary Public in and for said State, personally appeared Robert C. Baker, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as President of MIDDLESEX REALTY CORP., general partner of 46.24 ASSOCIATES, INC., and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Wayne E. Heller
Notary Public

WAYNE E. HELLER
Notary Public, State Of New York
No. 02HE6062858
Qualified In Westchester County
Commission Expires August 20, 2009

STATE OF NEW JERSEY)
} SS.:
COUNTY OF MIDDLESEX)

On the 21st day of September in the year 2006 before me, the undersigned, a Notary Public in and for said State, personally appeared Mark Boulding, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as SVPE General Counsel of **PTC THERAPEUTICS, INC.**, and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Sonja K. Benson
Notary Public

SONJA K. BENSON
NOTARY PUBLIC OF NEW JERSEY
Commission Expires 12/17/2007

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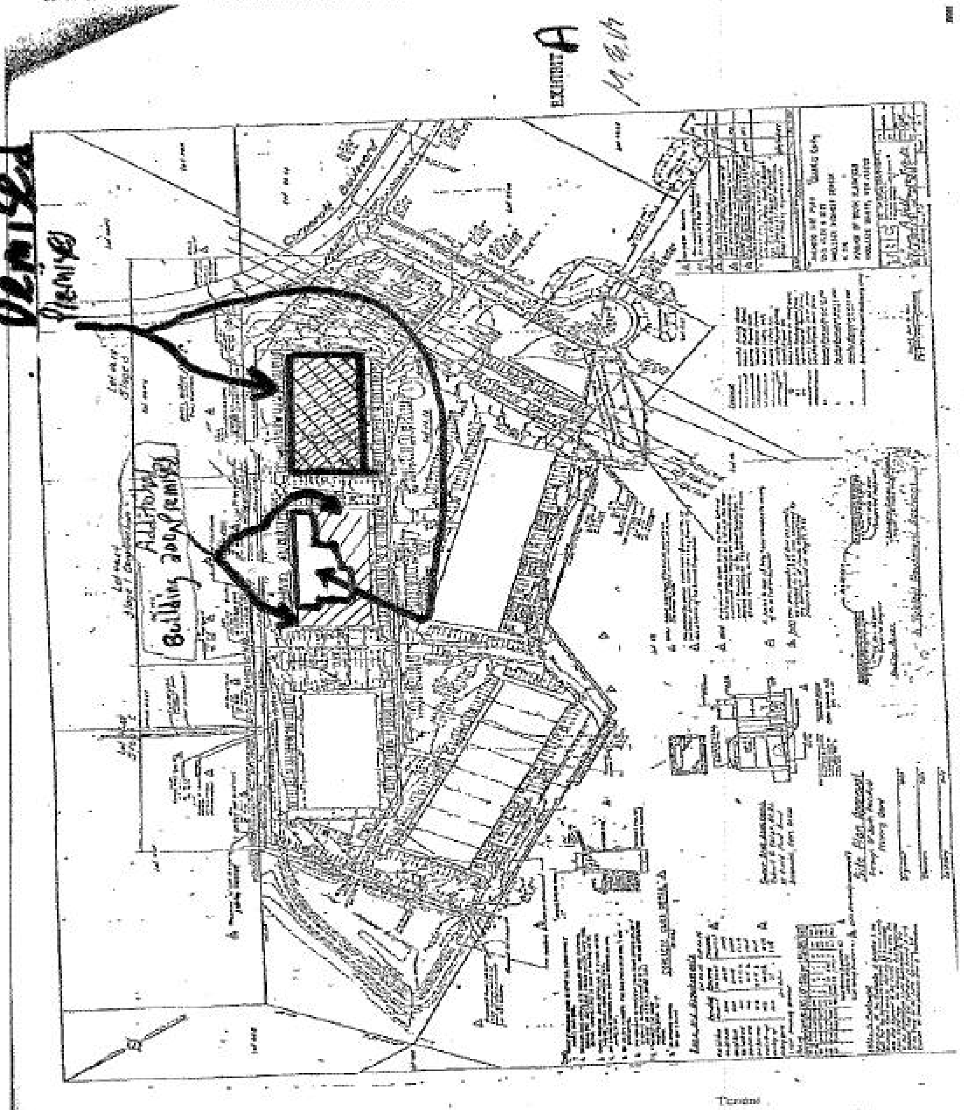
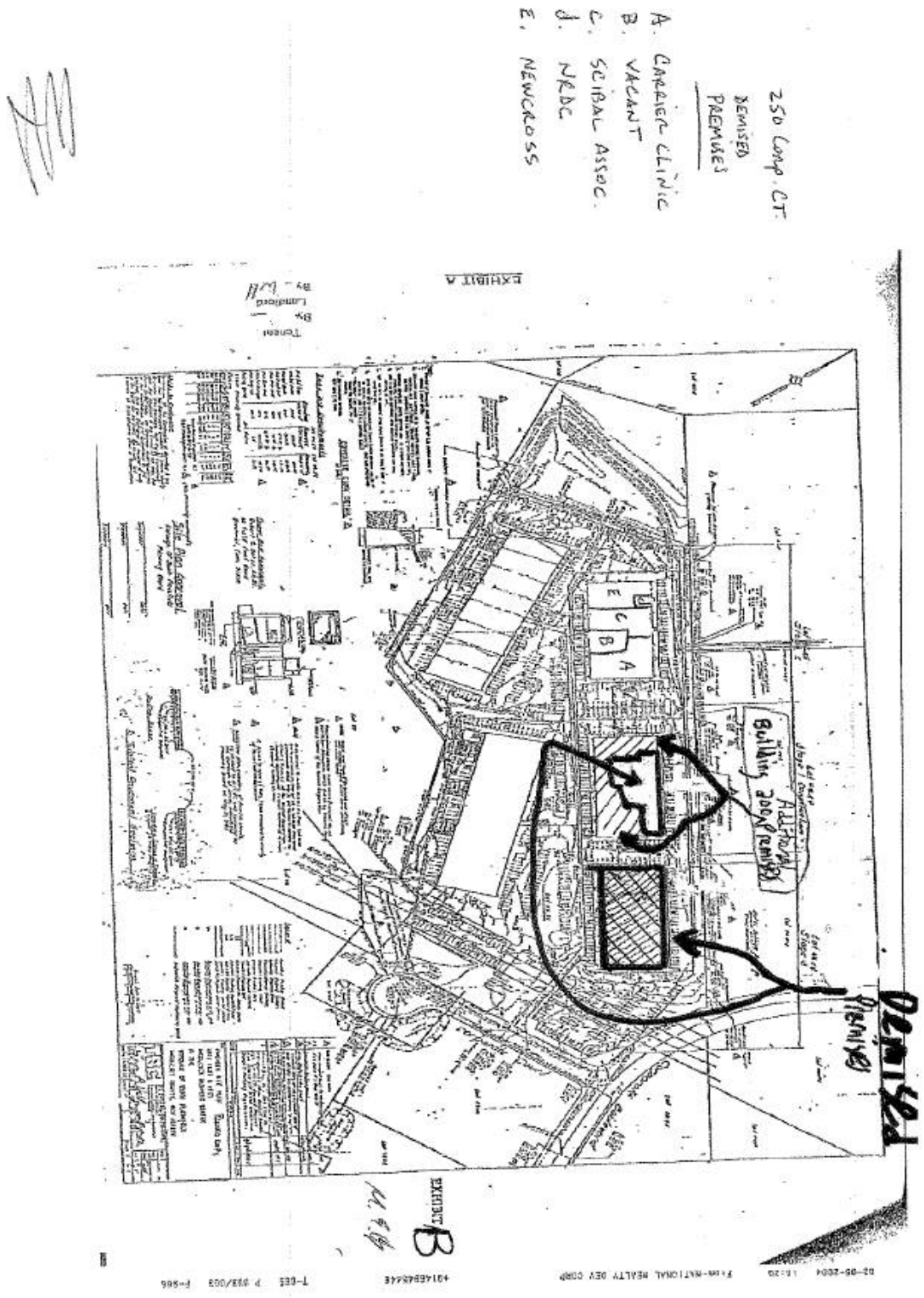


Tenant
By ___
Landlord
By ___

EXHIBIT A



THIRD MODIFICATION OF LEASE

This THIRD MODIFICATION OF LEASE (this "Modification") is made as of the 31st day of December 2008 by and between **46.24 ASSOCIATES L.P.,** a Delaware limited partnership, having its office and P.O. Address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord"), and **PTC THERAPEUTICS, INC.** a Delaware corporation, having an office at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (hereinafter referred to as "Tenant").

W I T N E S S E T H:

WHEREAS, Landlord and Tenant entered into that certain lease (herein referred to as the "Lease") dated as of July 11, 2000, as amended by letter agreements dated July 10, 2000 and December 14, 2000, by which Tenant leased from Landlord, and Landlord leased to Tenant, certain premises in building #100 of the Middlesex Business Center (the "Center") in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX, and STATE OF NEW JERSEY, which premises consist of approximately 21,700 square feet and are more particularly described in the Lease; and

WHEREAS, Landlord and Tenant entered into that certain First Modification and Extension of Lease Agreement dated December 1, 2003 whereby the Demised Premises were increased by 8,300 square feet in building #100 (bringing the total leased space in building #100 to 30,000 square feet), as well as adding 11,500 square feet of space located in Building #200, such that the Demised Premises then contained 41,500 square feet.

WHEREAS, Landlord and Tenant entered into that certain Second Modification of Lease dated October 20, 2006 whereby the Demised Premises were increased by 18,500 square feet in building #200 (bringing the total leased space in building #200 to 30,000 square feet), such that the Demised Premises now contain 60,000 square feet.

WHEREAS, Landlord and Tenant now desire to modify the Lease in certain respects in order to expand the Demised Premises;

NOW, THEREFORE, for TEN and 00/100 ($10.00) DOLLARS and other good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, the parties hereto agree as follows:

1. Tenant hereby leases from Landlord, and Landlord hereby leases to Tenant, the approximately 11,171 additional square feet of floor space in Building #250 more particularly indicated as Unit E on the plot plan annexed hereto as "Exhibit A" and hereby made a part hereof, (being Exhibit B from the Second Modification of Lease, showing the units available for rent to Tenant upon certain terms and conditions set forth in Section 8 of the Second Modification).

The "Effective Date" shall be the day Landlord delivers the "Building #250 Additional Premises- Unit E" to Tenant in broom clean condition (including removal of all previous tenants' fixtures, furnishings and equipment, data center cabling, Liebert air handlers, data center UPS, and office furniture), which Landlord shall do within two weeks of the date of this Modification ("Landlord's Work"). The Tenant will be responsible for removing the computer floor itself, the supporting structure for the computer floor and any fixtures or equipment below the floor.

Such 11,171 square feet of floor space is hereinafter referred to as the "Building #250 Additional Premises- Unit E". From and after the Effective Date, the Additional Premises shall be deemed to be part of the Demised Premises, as if the same was included within the Demised Premises as the same is defined by the Lease (so that from and after the Effective Date the Demised Premises shall consist of approximately 71,171 square feet). From and after the Effective Date, the plot plan attached to the Second Modification of Lease as Exhibit A thereto shall be deemed deleted from the Lease and the plot plan attached as Exhibit A to this Modification shall be deemed substituted therefore.

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2. Tenant has examined the Building #250 Additional Premises- Unit E and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the Building #250 Additional Premises- Unit E, except as is in this Modification specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to its knowledge, without independent investigation, that except as disclosed in the Phase I Environmental Assessment prepared by Environmental Management Services dated June 19, 2000, there are no environmental hazards present within the Building #250 Additional Premises. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation and examination, and Tenant accepts the Building #250 Additional Premises- Unit E in "as is" condition.

3. Tenant shall be responsible for performing such work as is needed so that the interior portions of the Building #250 Additional Premises- Unit E are compliant with the ADA. Landlord represents that it has performed such work as is needed so that the exterior portion of the Building #250 Additional Premises- Unit E (including all exterior portions of Building #250 itself) are compliant with the Americans with Disabilities Act (the "ADA").

4. Beginning on the Effective Date, the annual minimum rental payable under Section 3.01 of the Lease, as amended by Section 6 of the First Modification of Lease, Section 4 of the Second Modification of Lease, and this Modification, shall be as follows, notwithstanding anything in Section 3.01 of the Lease to the contrary:

> From the Effective Date and thereafter through December 31, 2009: **SIX HUNDRED SEVENTY SIX THOUSAND ONE HUNDRED TWENTY FOUR AND 50/100 ($676,124.50) DOLLARS per annum — FIFTY SIX THOUSAND THREE HUNDRED FORTY THREE AND 71/100 ($56,343.71) DOLLARS per month; and**
>
> From January 1, 2010 through February 28, 2012: **SEVEN HUNDRED TWENTY NINE THOUSAND FIVE HUNDRED TWO AND 75/100 ($729,502.75) DOLLARS per annum — SIXTY THOUSAND SEVEN HUNDRED NINETY ONE AND 90/100 ($60,791.90) DOLLARS per month.**

5. Effective immediately, Section 40.02 of the Lease (as modified by the First Modification and Extension of Lease, and Section 5 of the Second Modification of Lease) is hereby deleted and replaced with the following in its entirety.

Section 40.02. If Tenant shall duly elect to extend the Term as herein provided, the annual minimum rental payable by Tenant during said extension term shall be **EIGHT HUNDRED TWO THOUSAND EIGHT HUNDRED EIGHT AND 88/100 ($802,808.88) DOLLARS** per annum— **SIXTY SIX THOUSAND NINE HUNDRED AND 74/100 ($66,900.74) DOLLARS** per month.

6. Tenant may at any time elect to improve any of such leased space in Building #250, in accordance with Tenant's plans and specifications which shall be approved by Landlord in advance pursuant to the terms of the Lease (such approval not to be unreasonably withheld, conditioned or delayed), in which case Tenant shall (i) provide Landlord with proof of payment (in the form of copies of paid invoices) to all contractors, laborers, and suppliers involved with the Improvements, (ii) provide Landlord with lien waivers from all contractors, laborers and suppliers involved with the Improvements, and (iii) provide Landlord with a copy of the Certificate of Occupancy or applicable building inspector sign-offs covering the improved portion of the Demised Premises upon completion of the Improvements, along with "as built" plans. In such event, Landlord shall provide Tenant with a rent credit for amounts expended by Tenant for the construction of the Improvements in Building #250, in an amount not to exceed the sum of TWELVE ($12) Dollars per square foot of space so improved (the "Building #250 Allowance"), provided that, the Building #250 Allowance shall be adjusted downward by an amount equal to 20% for each full (but not partial) twelve-month period which has lapsed since January 1, 2007 and the Effective Date (except that in the event the Effective Date shall occur after December 31, 2008, it shall be deemed for the purposes of this Section, in order to calculate

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the Building #250 Allowance, to have occurred on December 31, 2008). Provided that Tenant is not in default of the terms of this Lease, such reimbursement shall be in the form of a credit to be taken by Tenant against the payment of annual minimum rental due and payable (immediately following Tenant's prior compliance with the requirements of this paragraph) which shall be credited in alternating months against 100% of such alternating monthly payments until exhausted. The Building #250 Allowance shall only be applied towards approved Improvements in Building #250, and shall not be applied towards Tenant's equipment, fixtures, inventory or other personal property charges.

Tenant shall not be entitled to any additional sums from Landlord in the event that the monies expended by Tenant in connection with the completion of the Improvements in Building #250 exceed the amount of the Building #250 Allowance.

7. Tenant covenants, warrants and represents that it has dealt with no broker other than Colliers Houston & Co. respecting this Modification and that no conversations, correspondence or negotiations were had with any broker (except with Colliers Houston & Co.) concerning the renting or leasing of the Building #250 Additional Premises- Unit E. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations with any broker except said Colliers Houston & Co. regarding the Lease. Landlord shall pay any commissions owing to said Colliers Houston & Co. with respect to this Modification in accordance with separate agreement.

8. Upon execution of this Modification by Tenant, Tenant shall deliver to Landlord adequate and proper insurance policies with respect to the Building #250 Additional Premises-Unit E including, without limitation, comprehensive general liability insurance in the amounts specified in Article 7 of this Lease.

Upon and after the execution of this Modification, Tenant shall have full and immediate access to the leased Building #250 premises to plan and commence architectural, engineering and construction activities, subject to Landlord's prior receipt of insurance certificates as set forth above, and provided that Tenant's activities do not interfere with Landlord's Work.

9. Except as expressly modified herein, all of the terms and conditions of the Lease shall continue unmodified and in full force and effect. Capitalized terms used herein without definition shall have the meaning given to such terms in the Lease. Obligations under this Agreement shall be deemed obligations under the Lease and that a default hereunder shall constitute a default under the Lease.

IN WITNESS WHEREOF, the parties have hereunto set their hands and seals as of the day and year first above written.

WITNESS:

[ILLEGIBLE]

46.24 ASSOCIATES L.P., a Delaware limited partnership

By: Middlesex Realty Corp., general partner

By: /s/ Robert C. Baker
Name: Robert C. Baker
Title: President

(LANDLORD)

[ILLEGIBLE]

PTC THERAPEUTICS, INC., a Delaware corporation

By: /s/ Mark E. Boulding
Name: **MARK E. BOULDING**
Title: **SVP & General Counsel**

(TENANT)

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L [ILLEGIBLE]

State of New York
} SS:
County of Westchester

On the 9th day of February in the year 2008 before me, the undersigned, a Notary Public in and for said State, personally appeared ROBERT C. BAKER personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as President of MIDDLESEX REALTY CORP., general partner of **46.24 ASSOCIATES, INC.,** and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ [ILLEGIBLE]
Notary Public

RICHARD A. KAUFMAN
Notary Public, State of New York
No. 4875196
Qualified in Westchester County
Commission Expires October 6, 2010

STATE OF NEW JERSEY)
} SS.:
COUNTY OF MIDDLESEX)

On the 31st day of December in the year 2008 before me, the undersigned, a Notary Public in and for said State, personally appeared Mark Boulding, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and

acknowledged to me that he executed the same in his capacity as SVP & Gen. Counsel of **PTC THERAPEUTICS, INC.,** and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Sonja K. Benson
Notary Public

Sonja K. Benson
Notary Public
New Jersey
Comm. Expires 12/17/2012

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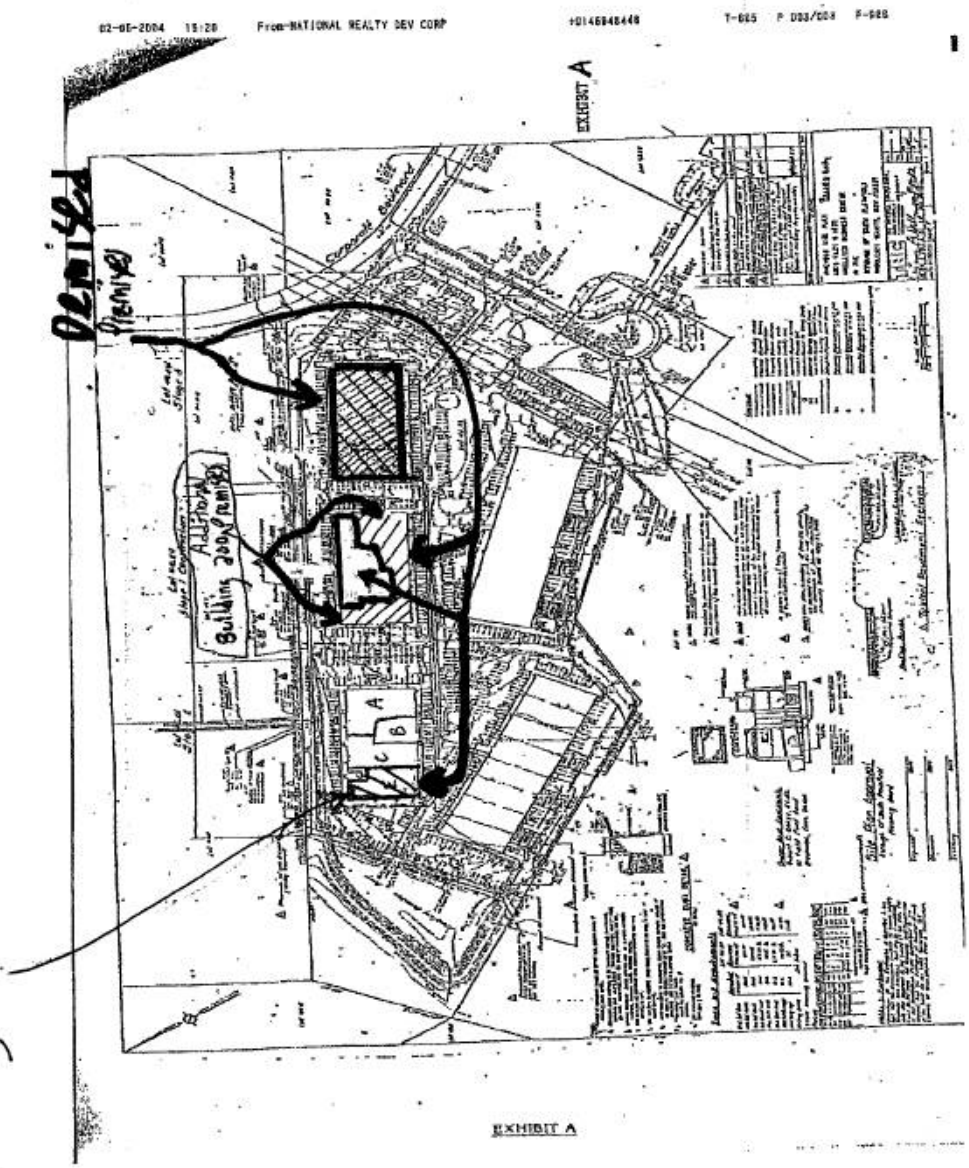
L [ILLEGIBLE]

Exhibit A



Building #250 Additional Premises - Unit E

250 Comp. CT.
DEMISED PREMISES

A. CHARTER CLINIC
B. VACANT
C. SCIBAL ASSOC.
D. NRDC
E. NEWCROSS

L
T

This FOURTH MODIFICATION OF LEASE (this "Modification") is made as of the 4th day of August, 2009 by and between **46.24 ASSOCIATES L.P.,** a Delaware limited partnership, having its office and P.O. Address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord"), and **PTC THERAPEUTICS, INC.** a Delaware corporation, having an office at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (hereinafter referred to as "Tenant").

W I T N E S S E T H:

WHEREAS, Landlord and Tenant entered into that certain lease (herein referred to as the "Lease") dated as of July 11, 2000, as amended by letter agreements dated July 10, 2000 and December 14, 2000, by which Tenant leased from Landlord, and Landlord leased to Tenant, certain premises in building #100 of the Middlesex Business Center (the "Center") in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX, and STATE OF NEW JERSEY, which premises consist of approximately 21,700 square feet and are more particularly described in the Lease; and

WHEREAS, Landlord and Tenant entered into that certain First Modification and Extension of Lease Agreement dated December 1, 2003 whereby the Demised Premises were increased by 8,300 square feet in building #100 (bringing the total leased space in building #100 to 30,000 square feet), as well as adding 11,500 square feet of space located in Building #200, such that the Demised Premises then contained 41,500 square feet; and

WHEREAS, Landlord and Tenant entered into that certain Second Modification of Lease Agreement dated October 20, 2006 whereby the Demised Premises were increased by 18,500 square feet in building #200 (bringing the total leased space in building #200 to 30,000 square feet), such that the Demised Premises then contained 60,000 square feet; and

WHEREAS, Landlord and Tenant entered into that certain Third Modification of Lease Agreement dated December 31, 2008 (the "Third Modification") whereby the Demised Premises were increased by 11,171 square feet in building #250 such that the Demised Premises now contain 71,171 square feet.

WHEREAS, Landlord and Tenant now desire to modify the Lease in certain respects;

NOW, THEREFORE, for TEN and 00/100 ($10.00) DOLLARS and other good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, the parties hereto agree as follows:

1. Tenant hereby leases from Landlord, and Landlord hereby leases to Tenant, for a term commencing on July 1, 2010 (the "Effective Date") and ending on February 29, 2012 (the "Expiration Date") approximately 11,627 additional square feet of floor space in Building #250 more particularly indicated and described by cross hatching on the plot plan annexed hereto as "Exhibit A" and hereby made a part hereof (such 11,627 square feet of floor space is hereinafter referred to as the "4D Premises"). From and after the Effective Date, the 4D Premises shall be deemed to be part of the Demised Premises, as if the same was included within the Demised Premises as the same is defined by the Lease (so that from and after the Effective Date the Demised Premises shall consist of approximately 82,798 square feet, 22,798 of which are located in separate non contiguous units within Building #250). From and after the Effective Date, the plot plan attached to the Lease as Exhibit A thereto shall be deemed deleted from the Lease and the plot plan attached as Exhibit A to this Modification shall be deemed substituted therefore.

2. Tenant has examined the 4D Premises and has made a complete inspection of same and is familiar with the physical condition thereof. Landlord has not made and does not make any representation as to the physical condition or any other matter affecting or relating to the 4D Premises, except as is in this Modification specifically set forth, and Tenant specifically acknowledges that no such representation has been made, except that Landlord represents, to its knowledge, without independent investigation, that except as disclosed in the Phase I

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Environmental Assessment prepared by Environmental Management Services dated June 19th, 2000, there are no environmental hazards present within the 4D Premises. Tenant further acknowledges that Landlord has afforded Tenant the opportunity for a full and complete investigation, examination, and inspection of the 4D Premises and Tenant accepts the 4D Premises "as is", except that Landlord hereby covenants and warrants that the 4D Premises shall be delivered in a similar condition to its condition as of the date of this Modification, subject to normal wear and tear.

3. Tenant shall be responsible for performing such work as is needed so that the interior portions of the 4D Premises are compliant with the Americans with Disabilities Act (the "ADA"). Landlord represents that it has performed such work as is needed so that the exterior portion of the 4D Premises (including all exterior portions of Building #250 itself) are compliant with the ADA.

4. Beginning on the Effective Date, the annual minimum rental payable under Section 3.01 of the Lease, as amended by Section 6 of the First Modification of Lease, Section 4 of the Second Modification of Lease, Section 4 of the Third Modification of Lease, and this Modification, shall be as follows, notwithstanding anything in Section 3.01 of the Lease to the contrary:

From Jury 1, 2010 through February 28, 2012: **EIGHT HUNDRED FORTY EIGHT THOUSAND SIX HUNDRED SEVENTY NINE AND 48/100 ($848,679.48) DOLLARS** per annum — **SEVENTY THOUSAND SEVEN HUNDRED TWENTY THREE and 29/100 ($70,723.29) DOLLARS** per month.

5. Effective immediately, Section 40.02 of the Lease (as modified by the First Modification and Extension of Lease, Second Modification of Lease, and Third Modification of Lease) is hereby deleted and replaced with the following quoted language in its entirety.

"Section 40.02. If Tenant shall duly elect to extend the Term as herein provided, the annual minimum rental payable by Tenant during said extension term shall be **NINE HUNDRED THIRTY THREE THOUSAND NINE HUNDRED SIXTY ONE AND 44/100 ($933,961.44) DOLLARS** per annum — **SEVENTY SEVEN THOUSAND EIGHT HUNDRED THIRTY AND 12/100 ($77,830.12) DOLLARS** per month."

6. For avoidance of doubt, Section 6 of the Third Modification, which relates to tenant improvements and related rental credits, shall continue to apply in accordance with its terms, such that Tenant shall receive a Credit of $4.80 per square foot of space in the 40 Premises.

7. Tenant covenants, warrants and represents that it has dealt with no broker other than Colliers Houston & Co. respecting this Modification and that no conversations, correspondence or negotiations were had with any broker (except with Colliers Houston & Co. concerning the negotiation of the Modification) concerning the renting or leasing of the 4D Premises. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations

with any broker except said Colliers Houston & Co. regarding the Lease. Landlord shall pay any commissions owing to said Colliers Houston & Co. with respect to this Modification in accordance with separate agreement

8. On or before the Effective Date, Tenant shall deliver to Landlord adequate and proper insurance policies with respect to the 4D Premises including, without limitation, comprehensive general liability insurance in the amounts specified in Article 7 of this Lease.

Upon reasonable request from time to time prior to the Effective Date, Tenant shall have reasonable access to the 4D Premises to plan architectural, engineering and construction activities, provided that Tenant's activities do not interfere with the current tenant's use and enjoyment of the 4D Premises. No construction activities shall actually be commenced unless and until Landlord's receipt of insurance certificates as set forth above and delivery of the 4D Premises to Tenant.

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9. Except as expressly modified herein, all of the terms and conditions of the Lease shall continue unmodified and in full force and effect. Capitalized terms used herein without definition shall have the meaning given to such terms in the Lease. Obligations under this Agreement shall be deemed obligations under the Lease and that a default hereunder shall constitute a default under the Lease.

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L [ILLEGIBLE]

IN WITNESS WHEREOF, the parties have hereunto set their hands and seals as of the day and year first above written.

WITNESS:

[ILLEGIBLE]

46.24 ASSOCIATES L.P., a Delaware limited partnership

By: Middlesex Realty Corp., general partner

By: /s/ Robert C. Baker
Name: Robert C. Baker
Title: President
(LANDLORD)

PTC THERAPEUTICS, INC., a Delaware corporation

[ILLEGIBLE]

By: /s/ Mark E. Boulding
Name: MARK E. BOULDING
Title: SVP & General Counsel
(TENANT)

State of New York
} SS:
County of Westchester

On the 11th day of August in the year 2009 before me, the undersigned, a Notary Public in and for said State, personally appeared **ROBERT C. BAKER** personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as President of MIDDLESEX REALTY CORP., general partner of 46.24 ASSOCIATES, INC., and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Wayne E. Heller
Notary Public

WAYNE E. HELLER
Notary Public, State Of New York
No. 02HE6062858
Qualified In Westchester County
Commission Expires August 20, 2009

STATE OF NEW JERSEY)
} SS.:
COUNTY OF MIDDLESEX)

On the 5 day of Aug in the year 2009 before me, the undersigned, a Notary Public in and for said State, personally appeared Mark Boulding, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as SrP,GC of **PTC THERAPEUTICS, INC.,** and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Jane Cavagnaro
Notary Public

JANE CAVAGNARO
NOTARY PUBLIC OF NEW JERSEY
I.D. # 2386244
Commission Expires 6/8/2014

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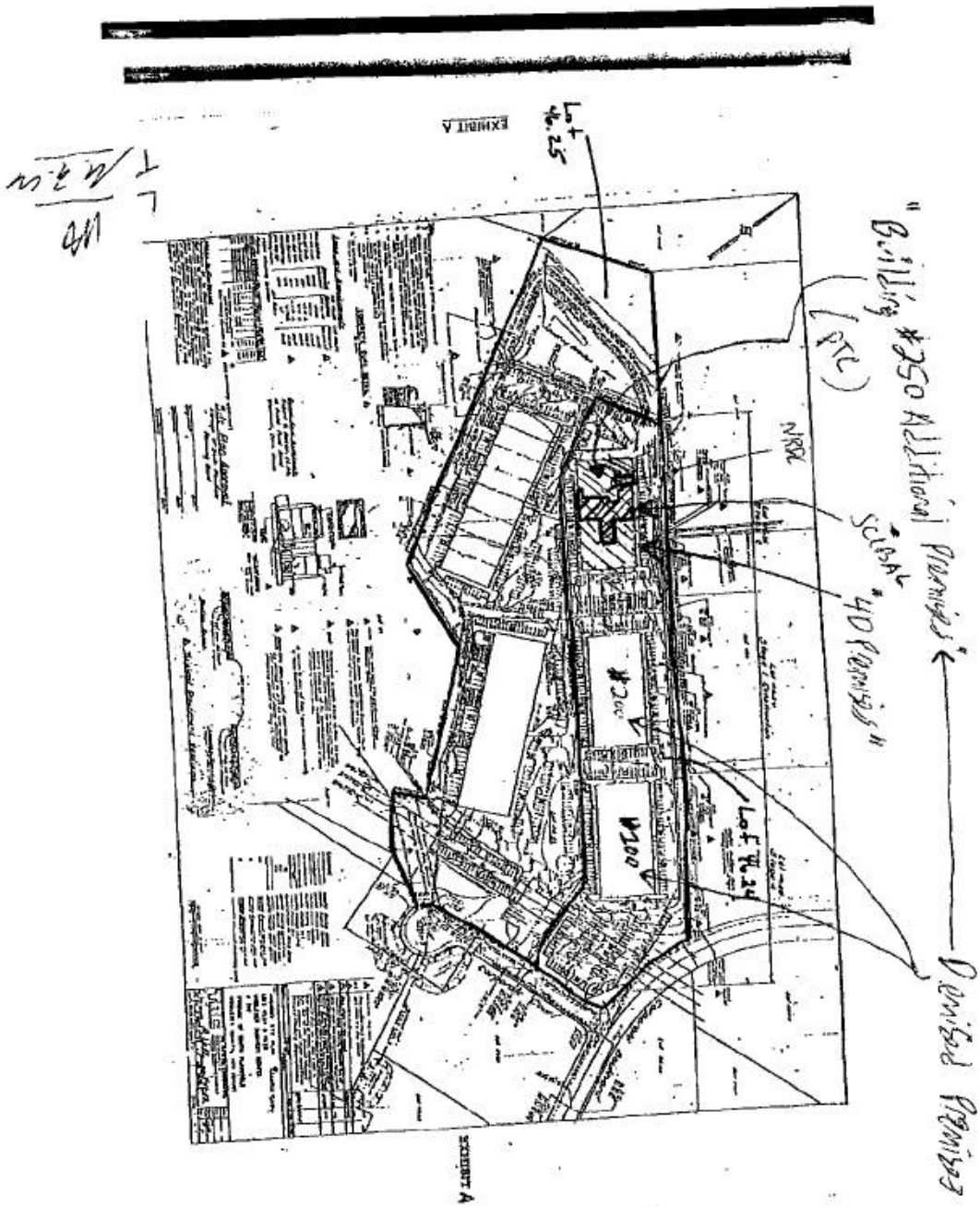

FIFTH MODIFICATION OF LEASE

This FIFTH MODIFICATION OF LEASE (this "Modification") is made as of the 10th day of June, 2011 by and between **46.24 ASSOCIATES L.P.,** a Delaware limited partnership, having its office and P.O. Address c/o National Realty & Development Corp., 3 Manhattanville Road, Purchase, New York 10577 (hereinafter referred to as "Landlord"), and **PTC THERAPEUTICS, INC.** a Delaware corporation, having an office at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (hereinafter referred to as "Tenant").

W I T N E S S E T H:

WHEREAS, Landlord and Tenant entered into that certain lease (herein referred to as the "Lease") dated as of July 11, 2000, as amended by letter agreements dated July 10, 2000 and December 14, 2000, by which Tenant leased from Landlord, and Landlord leased to Tenant, certain premises in building #100 of the Middlesex Business Center (the "Center") in the BOROUGH OF SOUTH PLAINFIELD, COUNTY OF MIDDLESEX, and STATE OF NEW JERSEY, which premises consisted of approximately 21,700 square feet and are more particularly described in the Lease; and

WHEREAS, Landlord and Tenant entered into that certain First Modification and Extension of Lease Agreement dated December 1, 2003 whereby the Demised Premises were increased by 8,300 square feet in building #100 (bringing the total leased space in building #100 to 30,000 square feet), as well as adding

11,500 square feet of space located in Building #200, such that the Demised Premises then contained 41,500 square feet; and

WHEREAS, Landlord and Tenant entered into that certain Second Modification of Lease Agreement dated October 20, 2006 whereby the Demised Premises were increased by 18,500 square feet in building #200 (bringing the total leased space in building #200 to 30,000 square feet), such that the Demised Premises then contained 60,000 square feet; and

WHEREAS, Landlord and Tenant entered into that certain Third Modification of Lease Agreement dated December 31, 2008 whereby the Demised Premises were increased by 11,171 square feet in building #250 such that the Demised Premises then contained 71,171 square feet; and

WHEREAS, Landlord and Tenant entered into that certain Fourth Modification of Lease Agreement dated August 4, 2009 whereby the Demised Premises were increased by 11,627 square feet in building #250 such that the Demised Premises now contain 22,798 feet in the building #250, and a total of 82,798 square feet at the Center; and

WHEREAS, the First Modification of Lease, Second Modification of Lease, Third Modification of Lease, and Fourth Modification of Lease are hereinafter referred to collectively as the "Modifications", and the Modifications and Lease are herein collectively referred to herein as the "Lease");

WHEREAS, the Term of the Lease is due to expire on February 29, 2012;

WHEREAS, Landlord and Tenant now desire to modify the Lease in certain respects;

NOW, THEREFORE, for TEN and 00/100 ($10.00) DOLLARS and other good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, the parties hereto agree as follows:

1. The Term of the Lease is hereby extended for a period of **SEVEN (7) YEARS** [MISSING COPY]

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acknowledges that there is no right to extend the Lease beyond the Expiration Date except as set forth in Section 3, below. The period commencing on March 1, 2012 and ending on the Expiration Date is herein referred to as the "Extended Term".

2. Notwithstanding anything to the contrary in the Lease, the annual minimum rental payable during the Extended Term under Section 3.01 of the Lease, shall be as follows:

From March 1, 2012 through February 28, 2018: **EIGHT HUNDRED FORTY EIGHT THOUSAND SIX HUNDRED SEVENTY NINE AND 48/100 ($848,679.48) DOLLARS per annum — SEVENTY THOUSAND SEVEN HUNDRED TWENTY THREE and 29/100 ($70,723.29) DOLLARS per month.**

From March 1, 2018 through February 28, 2019: **NINE HUNDRED SEVENTY TWO THOUSAND EIGHT HUNDRED SEVENTY SIX AND 48/100 ($972,876.48) DOLLARS per annum — EIGHTY ONE THOUSAND SEVENTY THREE and 04/100 ($81,073.04) DOLLARS per month.**

3. Article 40 of the Lease, as amended by the Modifications, is hereby deleted in its entirety and restated herein:

"Section 40.01 Tenant shall have the option, provided it is not in default hereunder, to extend the term of this Lease for TWO (2) successive additional terms of five (5) years each, upon the same terms and conditions as provided herein, except that the annual minimum rental during said extension period(s) shall be as provided below, and except that Tenant shall have no further extension options. Tenant shall give written notice to Landlord not less than nine (9) months prior to the last day of the Extended Term, or first extension term, as the case may be, of its election to extend the term hereof, or such option shall be deemed waived. If Tenant shall exercise such extension option, the parties will, at the request of either, execute an agreement in form for recording, evidencing such extension. If Tenant shall exercise such extension option, all references in this Lease to the term hereof shall be deemed to mean the term as so extended, except where expressly otherwise provided.

Section 40.02 If Tenant shall duly elect to exercise its option to extend the Term as herein provided, the annual minimum rental payable by Tenant, in the manner provided in Article 3 of the Lease, during said extension term, commencing on the first day of such extension term, shall be the product of ninety-five (95%) percent of the Fair Market Rent (hereinafter defined) for such extension term as determined in accordance with Section 40.03 below times 82,798 square feet (or if the Demised Premises shall have previously been altered and expanded or contracted in accordance with the terms of the Lease, then the then square footage of the Demised Premises).

Section 40.03 Tenant shall in the written notice by which Tenant exercises its extension option provided to it in this Article set forth Tenant's opinion of the Fair Market Rent (such opinion is herein referred to as "Tenant's FMR") and the basis therefor, provided, however, that if Tenant shall give Tenant's Election Notice more than eighteen (18) months in advance of the first day of the extension term, then Tenant shall not set forth Tenant's FMR in Tenant's Election Notice, but, rather shall set forth Tenant's FMR in a written notice given to Landlord no more than twelve (12) months prior to the first day of the extension term and no less than nine (9) months prior to the first day of the extension term. If Tenant shall not timely give Landlord written notice of Tenant's FMR, and such failure shall continue for more than ten (10) business days following written notice thereof from Landlord to Tenant of Tenant's failure to provide Landlord with the Tenant's FMR, then the Fair Market Rent shall promptly be determined solely by Landlord, and then Landlord shall promptly give Tenant written notice of same; provided, however, that Landlord's sole determination must be both reasonable and in good faith. If Tenant shall timely give Landlord written notice of Tenant's FMR, then Landlord shall have thirty (30) days after Landlord's receipt thereof (such thirty (30) day period is herein referred to as "Landlord's Review Period") to review the same. Within Landlord's Review Period Landlord shall provide Tenant with written notice advising Tenant whether or not Landlord accepts [MISSING COPY]

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accepted) Tenant's FMR, then the Fair Market Rent for the extension term in question shall be Tenant's FMR.

If Landlord shall not accept Tenant's FMR, then, within Landlord's Review Period, Landlord shall provide written notice of such non-acceptance to Tenant, together with Landlord's opinion of the Fair Market Rent (such opinion is herein referred to as "Landlord's FMR"), together with the reasons therefor. If Landlord shall not timely give Tenant written notice of Landlord's FMR, and such failure shall continue for more than ten (10) business days following written notice thereof from Tenant to Landlord of Landlord's failure to provide Tenant with Landlord's FMR, then the Fair Market Rent shall be deemed to be Tenant's

FMR, provided that Tenant's FMR has been determined in a reasonable and good faith manner. In the thirty (30) day period following Tenant's receipt of notice setting forth Landlord's FMR (such thirty (30) day period is herein referred to as the "Negotiating Period"), the parties shall negotiate, in good faith, to attempt to reach agreement on the Fair Market Rent.

If the parties reach agreement as to the Fair Market Rent within the Negotiating Period, then the Fair Market Rent shall be that agreed upon by the parties. If the parties are unable to reach agreement as to the Fair Market Rent within the Negotiating Period, then within ten (10) days of the lapse of the Negotiating Period (such ten (10) day period is herein referred to as the "Party's Selection Period") each party shall provide written notice to the other setting forth such party's then most recent opinion of the Fair Market Rent (such opinion set forth in Tenant's notice to Landlord is herein referred to as "Tenant's Final FMR" and such opinion set forth in Landlord's notice to Tenant is herein referred to as "Landlord's Final FMR") together with the name of an arbitrator selected by such party. The two arbitrators so selected shall then together, within fourteen (14) days after the lapse of the Party's Selection Period, select a third arbitrator (hereinafter referred to as the "third arbitrator") (and if either party shall fail to select an arbitrator (or fail to provide written notice of such selection to the other party) within the Party's Selection Period, then the remaining arbitrator shall alone select the third arbitrator). The third arbitrator shall then conduct such independent investigations as the third arbitrator may deem necessary to determine the Fair Market Rent, but the third arbitrator shall not consult with any of the arbitrators chosen by the parties hereto or with the parties hereto and shall not be provided with any of Tenant's FMR or Tenant's Final FMR or Landlord's FMR or Landlord's Final FMR. Within thirty (30) days of his/her selection as the third arbitrator, the third arbitrator shall give written notice of the third arbitrator's independent determination of the Fair Market Rent (such determination is herein referred to as the "Blind FMR") to each of the arbitrators selected by the parties hereto and to each of the parties hereto and the Fair Market Rent for the extension term in question shall be whichever of Tenant's Final FMR or Landlord's FMR is closest to the Blind FMR, provided that if Tenant's Final FMR and: Landlord's Final FMR shall be equidistant from the Blind FMR, then the Blind FMR shall be the Fair Market Rent for the extension term. Each party hereto shall pay the cost and expenses of the arbitrator selected by it and each party hereto shall be liable for one-half of the costs and expenses of such arbitrator. Each arbitrator, whether selected by a party hereto or by the arbitrators selected by the parties hereto, shall be a licensed real estate broker or MAI real estate appraiser having at least ten (10) year's experience in the Middlesex County (N.J.) commercial real estate market. The third arbitrator, in addition to the foregoing, shall be independent of the parties hereto and shall not have had any substantial business relationship, and shall not be employed by any entity having had a substantial business relationship, with any of the parties hereto.

Section 40.04 The "Fair Market Rent" with respect to the Demised Premises for the extension term shall be defined as the rental value of the Demised Premises as of the first day of the extension term expressed as an amount of money per square foot of the area of the Demised Premises determined as if the Demised Premises were available in the then-current rental market for space that tenants of similar credit standing as Tenant are leasing for non-subleased, non-encumbered, non-equity, office/laboratory space in a comparable building and having amenities similar to those located on or associated with the Demised Premises (such as on-site parking) for a term of five (5) years which other space is comparable in age, size, location, floor level and quality to the Demised Premises (including, without limitation, a comparable level of finishes

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4. Landlord hereby grants Tenant a non-exclusive revocable (as set forth in this paragraph, or due to Tenant's uncured breach of the Lease) license to pass through the "Scibal Premises" as shown on **Exhibit A** attached hereto and incorporated herein in order to pass to and from the "4D Premises" and the "Building 250 Additional Premises" which represent two (2) non-contiguous portions of the Demised Premises. The Scibal Premises shall only be used for convenient interior circulation by Tenant and its employees between these two units, and may not be used for storage, a break or smoking area, loitering, or any other use. If Landlord shall lease the Scibal Premises to another party then Landlord may terminate the license upon notice to Tenant. Prior to Tenant's use of the license, Tenant shall provide Landlord with evidence that Tenant maintains insurance that satisfies the requirements of the Lease which includes coverage for the Scibal Premises. Tenant's use of the license area shall be subject to Tenant's prior installation of lighting for security and safety, following Landlord's approval of the design and method of installation. Such lighting shall be on at all hours that persons are in the Demised Premises, or shall be activated by motion sensor whenever someone enters the Scibal Premises, but in either case, shall be installed by Tenant at its sole cost and expense and shall be wired to Tenant's electric account(s). Landlord reserves the right to use the Scibal Premises at any time for storage, to access same at any time for repairs, or to show prospective tenants.

As a condition to Tenant's use of the license through the Scibal Premises, Tenant shall install a door leading from each of the 4D Premises and the Building 250 Additional Premises to the Scibal Premises. Upon the expiration or earlier termination of the Lease, Tenant shall remove the doors and restore the adjacent walls to their original condition. Tenant shall secure its new doors with regard to access to/from the Scibal Premises as Tenant sees fit, acknowledging that Landlord has no obligation to ensure the security of the Scibal Premises and Landlord makes no representation or warranty as to the current or future security of the Scibal Premises (and thus the Demised Premises by means of the Scibal Premises).

5. Tenant covenants, warrants and represents that it has dealt with no broker other than Cassidy Turley New Jersey, Inc. f/k/a Colliers Houston & Co. respecting this Modification and that no conversations, correspondence or negotiations were had with any broker (except with Cassidy Turley f/k/a Colliers Houston & Co) concerning the renting or leasing of the 4D Premises. Tenant shall hold Landlord and National Realty & Development Corp. harmless and defend (by counsel satisfactory to Landlord) said parties against any claims for a brokerage commission arising out of any conversations, correspondence or negotiations with any broker except said Cassidy Turley f/k/a Colliers Houston & Co. regarding the Lease. Landlord shall pay any commissions owing to said Cassidy Turley f/k/a Colliers Houston & Co. with respect to this Modification in accordance with separate agreement.

6. Upon execution of this Modification by Tenant, Tenant shall deliver to Landlord adequate and proper insurance policies with respect to the Demised Premises, as well as the Scibal Premises, including, without limitation, comprehensive general liability insurance in the amounts specified in Article 7 of this Lease.

7. Except as expressly modified herein, all of the terms and conditions of the Lease, (including but not limited to §8, §9 and §11 of the 2nd Modification of Lease and §6 of the 3rd Modification of Lease), shall continue unmodified and in full force and effect. Capitalized terms used herein without definition shall have the meaning given to such terms in the Lease. Obligations under this Agreement shall be deemed obligations under the Lease and that a default hereunder shall constitute a default under the Lease.

8. The current Landlord shall provide Tenant with written notice of a proposed sale of Building 100 and/or Building 200, and/or Building 250, and any combination of such three buildings which shall not include any other buildings in Middlesex Business Center, at least fourteen (14) days prior to listing the sale with a real estate broker, or if such buildings are not then listed for sale, within fourteen (14) days of receipt of a written offer to purchase.

[remainder of the page intentionally left blank]

IN WITNESS WHEREOF, the parties have hereunto set their hands and seals as of the day and year first above written.

WITNESS:

/s/ Margo Ricci

[ILLEGIBLE]

46.24 ASSOCIATES L.P., a Delaware limited partnership

By: Middlesex Realty Corp., general partner

By: /s/ Brian Sekel
Name: Brian Sekel
Title: Vice President

(LANDLORD)

PTC THERAPEUTICS, INC., a Delaware corporation

By: /s/ Mark E. Boulding
Name: MARK E. BOULDING
Title: SVP & General Counsel

(TENANT)

State of New York
} SS:
County of Westchester

On the 10th day of June, in the year 2011 before me, the undersigned, a Notary Public in and for said State, personally appeared **BRIAN SEKEL** personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as Vice President of MIDDLESEX REALTY CORP., general partner of 46.24 ASSOCIATES, INC., and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Wayne E. Heller
Notary Public

WAYNE E. HELLER
Notary Public, State Of New York
No. 02HE6062858
Qualified In Westchester County
Commission Expires August 20, 2013

STATE OF NEW JERSEY)
} SS.:
COUNTY OF MIDDLESEX)

On the 27th day of May the year 2011 before me, the undersigned, a Notary Public in and for said State, personally appeared Mark E. Boulding, personally known to me or proved to me on the basis of satisfactory evidence to be the individual whose name is subscribed to the within instrument and acknowledged to me that he executed the same in his capacity as SVP & General Counsel of **PTC THERAPEUTICS, INC.,** and that by his signature on the instrument, the individual or the corporation upon behalf of which the individual acted, executed the instrument.

/s/ Paul Martin, Esq.
Notary Public

Paul Martin, Esq.
Notary Public - Union County
State of New Jersey
Commission Expires
August 4, 2014

OP ID: C8

CERTIFICATE OF LIABILITY INSURANCE

DATE (MM/DD/YYYY): 06/07/11

THIS CERTIFICATE IS ISSUED AS A MATTER OF INFORMATION ONLY AND CONFERS NO RIGHTS UPON THE CERTIFICATE HOLDER. THIS CERTIFICATE DOES NOT AFFIRMATIVELY OR NEGATIVELY AMEND, EXTEND OR ALTER THE COVERAGE AFFORDED BY THE POLICIES BELOW. THIS CERTIFICATE OF INSURANCE DOES NOT CONSTITUTE A CONTRACT BETWEEN THE ISSUING INSURER(S), AUTHORIZED REPRESENTATIVE OR PRODUCER, AND THE CERTIFICATE HOLDER.

IMPORTANT: If the certificate holder is an ADDITIONAL INSURED, the policy(ies) must be endorsed. If SUBROGATION IS WAIVED, subject to the terms and conditions of the policy, certain policies may require an endorsement. A statement on this certificate does not confer rights to the certificate holder in lieu of such endorsement(s).

PRODUCER Brown and Brown Metro Inc P.O. Box 679 Florham Park, NJ 07932-0679 Ken Udland 973-549-1900 973-549-1000	CONTACT NAME: Charlotte Lucas PHONE (A/C, No, Ext): 973-549-1915 FAX (A/C, No): E-MAIL ADDRESS: clucas@bbmetro.com PRODUCER CUSTOMER ID #: PTCTH-1
INSURED PTC Therapeutics Inc. 100 Corporate Court South Plainfield, NJ 07080	INSURER(S) AFFORDING COVERAGE — NAIC # INSURER A: Chubb Insurance Co. of NJ — 03299 INSURER B: Federal Insurance Co. — 20281 INSURER C: INSURER D: INSURER E: INSURER F:

COVERAGES **CERTIFICATE NUMBER:** **REVISION NUMBER:**

THIS IS TO CERTIFY THAT THE POLICIES OF INSURANCE LISTED BELOW HAVE BEEN ISSUED TO THE INSURED NAMED ABOVE FOR THE POLICY PERIOD INDICATED. NOTWITHSTANDING ANY REQUIREMENT, TERM OR CONDITION OF ANY CONTRACT OR OTHER DOCUMENT WITH RESPECT TO WHICH THIS CERTIFICATE MAY BE ISSUED OR MAY PERTAIN, THE INSURANCE AFFORDED BY THE POLICIES DESCRIBED HEREIN IS SUBJECT TO ALL THE TERMS, EXCLUSIONS AND CONDITIONS OF SUCH POLICIES. LIMITS SHOWN MAY HAVE BEEN REDUCED BY PAID CLAIMS.

INSR LTR	TYPE OF INSURANCE	ADDL INSR	SUBR WVD	POLICY NUMBER	POLICY EFF (MM/DD/YYYY)	POLICY EXP (MM/DD/YYYY)	LIMITS		
A	GENERAL LIABILITY X COMMERCIAL GENERAL LIABILITY CLAIMS-MADE X OCCUR GEN'L AGGREGATE LIMIT APPLIES PER: X POLICY PRO-JECT LOC	X		35830014	10/11/10	10/11/11	EACH OCCURRENCE DAMAGE TO RENTED PREMISES (Ea occurrence) MED EXP (Any one person) PERSONAL & ADV INJURY GENERAL AGGREGATE PRODUCTS - COMP/OP AGG	$ $ $ $ $ $	1,000,000 1,000,000 10,000 1,000,000 2,000,000 Excluded
B	AUTOMOBILE LIABILITY ANY AUTO ALL OWNED AUTOS SCHEDULED AUTOS X HIRED AUTOS X NON-OWNED AUTOS			73530569 73530569	10/11/10 10/11/10	10/11/11 10/11/11	COMBINED SINGLE LIMIT (Ea accident) BODILY INJURY (Per person) BODILY INJURY (Per accident) PROPERTY DAMAGE (Per accident)	$ $ $ $	1,000,000
A	UMBRELLA LIAB X OCCUR EXCESS LIAB CLAIMS-MADE DEDUCTIBLE RETENTION $			79836685	10/11/10	10/11/11	EACH OCCURRENCE AGGREGATE	$ $	5,000,000 5,000,000
A	WORKERS COMPENSATION AND EMPLOYERS' LIABILITY ANY PROPRIETOR/PARTNER/EXECUTIVE OFFICER/MEMBER EXCLUDED? Y/N (Mandatory in NH) If yes, describe under DESCRIPTION OF OPERATIONS below	N/A		1071723778	06/16/10	06/16/11	X WC STATU-TORY LIMITS OTH-ER E.L. EACH ACCIDENT E.L. DISEASE - EA EMPLOYEE E.L. DISEASE - POLICY LIMIT	$ $ $	1,000,000 1,000,000 1,000,000

DESCRIPTION OF OPERATIONS / LOCATIONS / VEHICLES (Attach ACORD 101, Additional Remarks Schedule, if more space is required)
Re: 100, 200, 250 Middlesex Business Center South Plainfield, NJ
Certholder (landlord) is included as additional insured as per lease of premises agreement.

CERTIFICATE HOLDER

46.24 Associates L.P.
c/o National Realty & Dev Corp
3 Manhattanville Rd
Purchase, NY 10577

CANCELLATION

SHOULD ANY OF THE ABOVE DESCRIBED POLICIES BE CANCELLED BEFORE THE EXPIRATION DATE THEREOF, NOTICE WILL BE DELIVERED IN ACCORDANCE WITH THE POLICY PROVISIONS.

AUTHORIZED REPRESENTATIVE

Charlotte Lucas

Exhibit 10.14

Final

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

License and Collaboration Agreement

This Agreement is entered into as of November 23, 2011 ("**Execution Date**") with effect as of the Effective Date (as defined below)

by and among

F. Hoffmann-La Roche Ltd

a Swiss corporation with an office and place of business at Grenzacherstrasse 124, 4070 Basel, Switzerland ("**Roche Basel**")

and

Hoffmann-La Roche Inc.

a New Jersey corporation with an office and place of business at 340 Kingsland Street, Nutley, New Jersey 07110, U.S.A. ("**Roche Nutley**"; Roche Basel and Roche Nutley together referred to as "**Roche**")

on the first hand

and

PTC Therapeutics, Inc.

a Delaware corporation with an office and place of business at 100 Corporate Court, South Plainfield, New Jersey 07080 ("**PTC**")

on the second hand

and (solely with respect to the Foundation Provisions (as defined below))

Spinal Muscular Atrophy Foundation

with its principal place of business at 888 Seventh Avenue, Suite 400, New York, New York 10019 ("**Foundation**")

on the third hand.

Table of Contents

License and Collaboration Agreement

WHEREAS, PTC has discovered proprietary compounds that are potentially useful for the treatment of spinal muscular atrophy and possesses proprietary technology and intellectual property rights relating thereto; and

WHEREAS, in cooperation with the Foundation, PTC has initiated development of these compounds; and

WHEREAS, Roche has expertise in the research, development, manufacture and commercialization of pharmaceutical and diagnostic products; and

WHEREAS, PTC and Roche desire to collaborate on the discovery, research, development and commercialization of products containing such compounds; and

WHEREAS, Foundation is willing to assist in such collaboration by agreeing to the Foundation Provisions of this Agreement;

NOW, THEREFORE, in consideration of the mutual covenants and promises contained in this Agreement and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereto, intending to be legally bound, do hereby agree as follows:

1. Definitions

As used in this Agreement, the following terms, whether used in the singular or plural, shall have the following meanings:

1.1 Affiliate

The term "Affiliate" shall mean any individual, corporation, association or other business entity that directly or indirectly controls, is controlled by, or is under common control with the Party in question or the Foundation. As used in this definition of "Affiliate," the term "control" shall mean the direct or indirect ownership of more than fifty percent (>50%) of the stock having the right to vote for directors thereof or the ability to otherwise control the management of the corporation or other business entity whether through the ownership of voting securities, by contract, resolution, regulation or otherwise. Anything to the contrary in this paragraph notwithstanding, Chugai Pharmaceutical Co., Ltd, a Japanese corporation ("**Chugai**"), shall not be deemed an Affiliate of Roche unless Roche provides written notice to PTC of its desire to include Chugai as an Affiliate of Roche. Notwithstanding such written notice, if Chugai does not agree to be bound by the terms and conditions of this Agreement, then Chugai shall have none of the rights and obligations of an Affiliate of Roche under this Agreement.

1.2 Agreement

The term "Agreement" shall mean this document including any and all appendices and amendments to it as may be added and/or amended from time to time in accordance with the provisions of this Agreement.

1.3 Agreement Term

The term "Agreement Term" shall mean the period of time commencing on the Effective Date and, unless this Agreement is terminated sooner as provided in Article 20, expiring on the date when no royalty or other payment obligations under this Agreement are or will become due.

1.4 Alternative Splicing

The term "Alternative Splicing" shall mean an approach to developing therapeutics based on causing alternative splicing of a gene of interest (as a direct consequence of the therapy) to restore functionality of the related protein.

1.5 AS Assay

The term "AS Assay" shall mean PTC's proprietary technology for the identification of small molecules that cause Alternative Splicing of a gene of interest, including but not limited to SMN2.

1.6 Business Day

The term "Business Day" shall mean 9:00 a.m. to 5:00 p.m. local time on a day that is not a Saturday, Sunday or a day on which banking institutions in Newark, New Jersey or in Basel, Switzerland are authorized by Law to remain closed.

1.7 Calendar Quarter

The term "Calendar Quarter" shall mean each period of three (3) consecutive calendar months, ending March 31, June 30, September 30, and December 31.

1.8 Calendar Year

The term "Calendar Year" shall mean the period of time beginning on January 1 and ending December 31, except for the first year which shall begin on the Effective Date and end on December 31.

1.9 Change of Control

The term "Change of Control" shall mean, with respect to a Party: (a) the acquisition by any Third Party of beneficial ownership of fifty percent (50%) or more of the then outstanding common shares or voting power of such Party, other than (i) acquisitions by employee benefit plans sponsored or maintained by such Party, (ii) the initial public offering of a Party, or (iii) the acquisition by an institutional investor (or group of institutional investors), such as a venture capital fund, private equity fund or hedge fund, of shares of a Party for investment purposes in a transaction approved by such Party's Board of Directors; (b) the consummation of a business combination involving such Party (but not the other Party or any of its Affiliates), unless, following such business combination, the stockholders of such Party immediately prior to such business combination beneficially own directly or indirectly more than fifty percent (50%) of the then outstanding common shares or voting power of the entity resulting from such business

combination. For clarity, a transaction or series of related transactions pursuant to which a Party consolidates or merges with another entity and the holders of the outstanding voting shares of such Party immediately preceding such consolidation or merger hold more than fifty percent (50%) of the voting shares of the resulting entity (a "**Reverse Merger**") shall not be considered to be a Change of Control.

1.10 Clinical Study

The term "Clinical Study" shall mean a Phase I Study, Phase II Study, or Pivotal Study, as applicable.

1.11 Combination Product

The term "Combination Product" shall mean a Product that, in addition to containing a Compound as an active ingredient, also contains at least one other active pharmaceutical ingredient in a finished form.

1.12 Commercially Reasonable Efforts

The term "Commercially Reasonable Efforts" shall mean

(a) with respect to the efforts to be expended by a Person with respect to any objective, except as otherwise provided in clause (b) below, such reasonable, diligent and good faith efforts as such Person would normally use to accomplish a similar objective under similar circumstances by the specified deadline (if any) and within the specified budget (if any); and

(b) with respect to any objective relating to the research, development, manufacture or commercialization of a Compound or Product, the efforts and resources normally used by a company in the biopharmaceutical industry for a product which is of similar market potential at a similar stage in its development or commercialization, which level of effort is at least commensurate with the level of effort that a Party would devote to its own internally discovered and funded compounds or products that are of most closely comparable market potential at a most closely comparable stage in their development or product life, taking into account regulatory requirements of safety and efficacy, product profile, the competitiveness of the marketplace, the proprietary position of the product, and the cost of scaling up a manufacturing process (including facility costs), and the market potential of the applicable product.

1.13 Compound

The term "Compound" shall mean a New Compound or an SRA Compound.

1.14 Confidential Information

The term "Confidential Information" shall mean any and all information, data or know-how (including without limitation Know-How), whether technical or non-technical, oral or written, that is disclosed by one Party (or the Foundation) or its Affiliates ("Disclosing Party") to the other Party (or the Foundation) or its Affiliates ("Receiving Party"). Confidential Information shall not include any information, data or know-how that:

(i) was generally available to the public at the time of disclosure, or information that becomes available to the public after disclosure by the Disclosing Party other than through fault (whether by action or inaction) of the Receiving Party,

(ii) can be shown by cogent written records to have been already known to the Receiving Party prior to its receipt from the Disclosing Party,

(iii) is obtained by the Receiving Party at any time lawfully from a Third Party without obligations of non-use or non-disclosure,

(iv) is developed independently by the Receiving Party as evidenced by written records other than through knowledge of Confidential Information of the Disclosing Party, or

(v) is approved in writing by the Disclosing Party for release by the Receiving Party.

The terms of this Agreement and the SRA shall be considered Confidential Information of both Parties and Foundation.

1.15 Control

The term "Control" shall mean (as an adjective or as a verb including conjugations and variations such as "Controls" "Controlled" or "Controlling") (a) with respect to Patent Rights and/or Know-How, the possession by a Party of the ability to grant a license or sublicense of such Patent Rights and/or Know-How without violating the terms of any agreement or arrangement between such Party and any other Person and (b) with respect to proprietary materials, the possession by a Party of the ability to supply such proprietary materials to the other Party as provided herein without violating the terms of any agreement or arrangement between such Party and any other Person. Notwithstanding the foregoing, intellectual property of a Party that is licensed or otherwise acquired from any other Person after the Effective Date and would otherwise be considered to be under the Control of a Party shall not be deemed to be under the Control of such Party if the application of such definition in the context of any license grants or sublicenses under this Agreement would require the granting Party to make additional payments or royalties to any other Person in connection with such license or sublicense grants, unless the other Party agrees to pay the additional payments or royalties to the other Person.

1.16 Cover

The term "Cover" shall mean (as an adjective or as a verb including conjugations and variations such as "Covered," "Coverage" or "Covering") with respect to a product, composition, technology, process or method of use that, in the absence of ownership of or a license granted under a Valid Claim, the manufacture, use, offer for sale, sale or importation of such product or composition, or the practice of such technology, process or method of use would infringe such Valid Claim (or, in the case of a Valid Claim that has not yet issued, would infringe such Valid Claim if it were to issue). The determination of whether a product, composition, technology, process or method of use is Covered by a Valid Claim shall be made on a country-by-country basis.

1.17 Derivative

The term "Derivative" means, with respect to a specific chemical compound (the "Parent Compound"), (a) a structural variant of such Parent Compound in which one or more atoms or functional groups of the Parent Compound are replaced with different atoms or functional groups, and wherein such variant and

such Parent Compound have substantially similar core chemical structures and substantially similar biological activities, or (b) a metabolite, prodrug, solvate, ester, salt, stereoisomer, racemate, tautomer or polymorph of the Parent Compound.

1.18 Development Candidate Criteria

The term "Development Candidate Criteria" means the criteria the JSC uses to determine whether an SRA Drug Candidate is an SRA Development Candidate and/or whether a New Compound is a New Compound Development Candidate. The Development Candidate Criteria as of the Effective Date are attached hereto as Appendix 1.18.

1.19 Effective Date

The term "Effective Date" shall mean the HSR Clearance Date or, if no filing is to be made under the HSR Act, then it shall mean the Execution Date.

1.20 EMA

The term "EMA" shall mean the European Medicines Agency and any successor agency with responsibilities comparable to those of the European Medicines Agency.

1.21 EU

The term "EU" shall mean the European Union, as it may be redefined from time to time.

1.22 FBMC

The term "FBMC" shall mean, for a Product, the standard manufacturing cost, as defined by the manufacturing Party's standard cost accounting practices and policies and consistently applied by such Party. FBMC shall include direct labor, materials, product testing costs, including quality control and quality assurance bulk testing and in-process testing (e.g.,

adventitious virus and mycoplasma testing), and allocable overhead for manufacturing or contracting for each stage of the manufacturing process of the Product shipped. In addition, FBMC includes failures that are considered normal yield losses that could be reasonably expected or justified in this area of technology, excess capacity and idle plant cost to the extent associated with the Product, and write off and disposal costs of expired goods (raw materials, intermediates and products).

1.23 FDA

The term "FDA" shall mean the US Food and Drug Administration and any successor agency thereto.

1.24 FDCA

The term "FDCA" shall mean the US Food, Drug and Cosmetics Act.

1.25 Field

The term "Field" shall mean any and all uses.

1.26 Filing

The term "Filing" shall mean the filing of an application by the FDA as defined in the FDCA and applicable regulations, or the equivalent application to the equivalent agency in any other country or group of countries, the official approval of which is required before any lawful commercial sale or marketing of Products.

1.27 First SRA Amendment

The term "First SRA Amendment" shall mean Amendment No. 1 to the SRA, said First SRA Amendment having an effective date of October 12, 2007.

1.28 First Commercial Sale

The term "First Commercial Sale" shall mean, with respect to a Product in a country in the Territory, the first *bona fide* arms-length sale of such Product sold to a Third Party in such country by or on behalf of a member of the Roche Group after Regulatory Approval has been obtained for such Product in such country, or if no such Regulatory Approval is required, the date upon which Product is first commercially launched in such country.

1.29 Foundation Provisions

The term "Foundation Provisions" shall mean Sections 1.18, 1.29, 1.50, 1.69 (including Research Plan), 1.78, 1.82, 1.88 (including Transitional Research Plan), 4.1.3, 17.3, 20.5 (to the extent applicable to other Foundation Provisions), 22.5, 22.6, 22.7, 22.8, 22.9, 22.10, 22.11, 22.12, 22.13, 22.14, 22.15, 22.16 and Articles 1 (to the extent applicable to other Foundation Provisions), 2, 5, 7, 18 (last two sentences only) and 19.

1.30 Fourth SRA Amendment

The term “Fourth SRA Amendment” shall mean Amendment No. 4 to the SRA, said Fourth SRA Amendment having an effective date of November 22, 2011.

1.31 FTE

The term “FTE” shall mean the efforts of one or more employees of PTC equivalent to the efforts of one full-time PTC employee for one year.

1.32 FTE Rate

The term “FTE Rate” shall mean [**] dollars ($[**]) per FTE for the period commencing on the Effective Date and ending December 31, 2012. On January 1, 2013 and on January 1st of each subsequent Calendar Year, the foregoing rate shall be increased for the Calendar Year then commencing by the percentage increase, if any, in the Consumer Price Index (“CPI”) as of December 31 of the then most recently completed Calendar Year with respect to the level of the CPI on December 31, 2010. As used in this Section 1.32, Consumer Price Index or CPI means the Consumer Price Index — Urban Wage Earners and Clerical Workers, US City Average, All Items, 1982-84 = 100, published by the US Department of Labor, Bureau of Labor Statistics (or its successor equivalent index).

1.33 Generic Competition

The term “Generic Competition” shall mean, with respect to a Product in a country in the Territory in a given Calendar Quarter, if, during such Calendar Quarter, one or more Generic Products shall be commercially available in such country and such Generic Products shall have a market share of [**] percent ([**]%) or more of the aggregate market in such country of such Product and such Generic Products (based on sales of units of such Product and such Generic Products, as reported by IMS International, or if such data are not available, such other reliable data source as reasonably determined by Roche and agreed by PTC).

1.34 Generic Product

The term “Generic Product” shall mean, with respect to a Product in a country in the Territory, any pharmaceutical product sold by a Third Party not authorized by or on behalf of a member of the Roche Group that (a) contains as an active pharmaceutical ingredient the same Compound as the one contained in such Product, (b) is “a therapeutic equivalent” to such Product as such term is used in the Approved Drug Products with Therapeutic Equivalence Evaluations published by the FDA Center for Drug Evaluation and Research or any successor publication, and (c) is approved in reliance on the prior approval of such Product as determined by the applicable Regulatory Authority in such country.

1.35 Handle

The term “Handle” shall mean, with respect to a Patent Right, preparing, filing, prosecuting (including interference and opposition proceedings) and maintaining (including interferences, reissue, re-examination and opposition proceedings) such Patent Right.

1.36 HSR Act

The term “HSR Act” shall mean the US Hart Scott Rodino Antitrust Improvement Act.

1.37 HSR Clearance Date

The term “HSR Clearance Date” shall mean the earlier of (a) the date on which the FTC or DOJ shall notify PTC and Roche of early termination of the applicable waiting period under the HSR Act, or (b) the day after the date on which the applicable waiting period under the HSR Act expires; provided, however, if the FTC or DOJ shall commence any investigation by means of a second request or otherwise, HSR Clearance Date shall mean the date on which any investigation opened by the FTC or DOJ shall have been terminated, without action to prevent the Parties from implementing the transactions contemplated by this Agreement with respect to the US.

1.38 IFRS

The term “IFRS” shall mean International Financial Reporting Standards.

1.39 IND

The term “IND” shall mean an application as defined in the FDCA and applicable regulations promulgated by the FDA, or the equivalent application to the equivalent agency in any other country or group of countries, the filing of which is necessary to commence clinical testing of a Product in humans.

1.40 Initiation

The term “Initiation” shall mean the date that a human is first dosed with a Product in a Clinical Study approved by the respective Regulatory Authority.

1.41 Insolvency Event

The term “Insolvency Event” shall mean circumstances under which a Party (i) has a receiver or similar officer appointed over all or a substantial part of its assets or undertaking; (ii) passes a resolution for winding-up (other than a winding-up for the purpose of, or in connection with, any solvent amalgamation or reconstruction) or a court makes an order to that effect or a court makes an order for administration (or any equivalent order in any jurisdiction); (iii) ceases to carry on business; or (iv) is unable to pay its debts as they become due in the ordinary course of business.

1.42 Invention

The term “Invention” shall mean an invention that is conceived or reduced to practice in connection with any activity carried out pursuant to this Agreement. Under this definition, an Invention may be made by employees of PTC solely or jointly with a Third Party (a “PTC Invention”), by employees of the Roche Group solely or jointly with a Third Party (a “Roche

Invention"), or jointly by employees of PTC and of a member of the Roche Group with or without a Third Party (a "Joint Invention").

1.43 Joint Know-How

The term "Joint Know-How" shall mean Know-How that is made jointly by the Parties or their Affiliates or their Sublicensees in connection with any activity carried out pursuant to this Agreement.

1.44 Joint Patent Rights

The term "Joint Patent Rights" shall mean all Patent Rights Covering a Joint Invention.

1.45 Know-How

The term "Know-How" shall mean data, knowledge and information, including without limitation materials, samples, chemical manufacturing data, toxicological data, pharmacological data, preclinical and clinical data, assays, platforms, formulations, specifications, or quality control testing data, that are necessary or useful for the discovery, manufacture, development or commercialization of a Product.

1.46 Law

The term "Law" shall mean all laws, statutes, rules, regulations, orders, judgments, or ordinances having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.47 Major Countries

The term "Major Countries" shall mean US, UK, Germany, France, Italy, Spain, and Japan.

1.48 Marketing Exclusivity

The term "Marketing Exclusivity" shall mean, with respect to a Product in a country: (a) the exclusivity afforded to the Product for being the first drug product containing the active ingredient to receive Regulatory Approval in that country, (b) pediatric exclusivity, (c) orphan drug exclusivity, but only when all other indications of the applicable Product that have been approved by the applicable Regulatory Authority and appear in the labeling for such Product are also protected by an unexpired orphan exclusivity or other exclusivity pursuant to clause (a), (b) or (d) of this Section 1.48, or (d) other statutory and/or regulatory exclusivity.

1.49 NDA

The term "NDA" shall mean a new drug application, including all necessary documents, data, and other information concerning a Product, required for Regulatory Approval of the Product as a pharmaceutical product by the FDA, or an equivalent application to the equivalent

Regulatory Authority in any other country or group of countries (e.g., the marketing authorization application (MAA) filed with the EMA).

1.50 Net Sales

The term "Net Sales" shall mean, for a Product in a particular period, the amount calculated by subtracting from the Sales of such Product for such period: (i) a lump sum deduction of (A) [**] percent ([**]%) of Sales, with respect to Sales in the US, (B) [**] percent ([**]%) of Sales, with respect to Sales in the Major Countries (other than the US), Canada, Brazil and Switzerland, and (C) [**] percent ([**]%) of Sales, with respect to Sales in all territories other than those set forth in clauses (A) and (B) above, in lieu of those deductions that are not accounted for within Roche on a Product-by-Product basis (*e.g.*, freight, postage charges, transportation insurance, packing materials for dispatch of goods, custom duties); (ii) uncollectible amounts and credit card charges (including processing fees) accrued during such period on such Sales and not already taken as a gross-to-net deduction in accordance with the then currently used IFRS in the calculation of Sales of such Product for such period; and (iii) government mandated fees and taxes and other government charges accrued during such period on such Sales not already taken as a gross-to-net deduction in accordance with the then currently used IFRS in the calculation of Sales of such Product for such period, including, for example, any fees, taxes or other charges that become due in connection with any healthcare reform, change in government pricing or discounting schemes, or other action of a government or regulatory body. Notwithstanding the foregoing, amounts received by any member of the Roche Group for the sale of Product among members of the Roche Group for resale shall not be included in the computation of Sales. As expressly contemplated in the definition of "Worldwide Net Sales" set forth in the Second SRA Amendment, this definition of "Net Sales", together with the definition of "Sales" set forth in Section 1.78, shall be used in lieu of the Net Sales definition contained in the SRA, solely to calculate the "net sales by Licensee" pursuant to the SRA wherein such Licensee is Roche, for the sole purposes of determining the amount of "Worldwide Net Sales" pursuant to the SRA; for clarity, Sections 1.50 and 1.78 shall not otherwise be used with respect to the SRA and the definitions of "Net Sales" and "Worldwide Net Sales" as set forth in the SRA shall remain unchanged.

1.51 New Compound

The term "New Compound" shall mean any small molecule compound, other than an SRA Compound, that, as a direct result of its therapeutic application, results in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA, including without limitation compounds, other than SRA Compounds, first synthesized or identified by either Party in the Research Program, and any salts, polymorphs, esters, metabolites, pro-drugs, isomers and enantiomers thereof.

1.52 New Compound Development Candidate

The term "New Compound Development Candidate" means a New Compound that the JSC formally declares meets the Development Candidate Criteria, indicating that such New Compound is suitable for progression to IND-enabling pre-clinical studies in support of future human clinical trials.

1.53 New Product

The term "New Product" shall mean any product, including without limitation any Combination Product, containing a New Compound as a pharmaceutically active agent, regardless of its finished form, formulation or dosage.

1.54 New Product Development Plan

The term "New Product Development Plan" shall mean the plan for the development of New Products as set forth in Section 5.2.

1.55 Party

The term "Party" shall mean PTC or Roche, as the case may be, and "Parties" shall mean PTC and Roche collectively. For clarity, "Party" and "Parties" do not include or otherwise refer to Foundation.

1.56 Patent Rights

The term "Patent Rights" shall mean all rights under any patent or patent application, in any country of the Territory, including any patents issuing on such patent application, and further including any substitution, extension or supplementary protection certificate, reissue, reexamination, renewal, divisional, continuation or continuation-in-part of any of the foregoing.

1.57 Person

The term "Person" shall mean any natural person, corporation, general partnership, limited partnership, joint venture, proprietorship or other business organization or a governmental agency or a political subdivision thereto.

1.58 Phase I Study

The term "Phase I Study" shall mean any human clinical study of a Product that is intended as initial clinical safety testing in healthy volunteers or a limited patient population, or studies directed toward understanding the mechanisms or metabolism of the Product.

1.59 Phase II Study

The term "Phase II Study" shall mean any human clinical study of a Product subsequent to a Phase I Study and prior to a Pivotal Study that is intended to study the safety, dosage and initial efficacy in a limited patient population, and is prospectively designed to support the continued testing of the Product in one or more further Phase II Studies or in a Pivotal Study.

1.60 Pivotal Study

The term "Pivotal Study" shall mean a pivotal human clinical study of a Product that is prospectively designed to confirm with statistical significance in an expanded patient population the efficacy and safety of a drug in a given patient population, and the results of which are

intended to form the basis for Regulatory Approval. For the avoidance of doubt, a clinical trial that meets the foregoing criteria shall be deemed a Pivotal Study regardless of whether it is characterized as a "Phase 2b," or "Phase 2b/3," or "Phase 3" clinical trial.

1.61 Product

The term "Product" shall mean a New Product or an SRA Product.

1.62 Product Development Program

The term "Product Development Program" means the activities undertaken by the Parties pursuant to the SRA Development Plan to develop SRA Products and pursuant to the New Product Development Plan to develop New Products.

1.63 PTC Base Patent Rights

The term "PTC Base Patent Rights" shall mean any and all Patent Rights in the Territory that are Controlled by PTC as of the Effective Date and either Cover a Product or relate to the discovery, manufacture, development or commercialization of a Product, said Patent Rights being exhaustively listed in Appendix 1.63 of this Agreement.

1.64 PTC Know-How

The term "PTC Know-How" shall mean the Know-How that PTC Controls at the Effective Date and during the Agreement Term.

1.65 PTC Patent Rights

The term "PTC Patent Rights" shall mean any and all Patent Rights that are Controlled by PTC during the Agreement Term and that either Cover a Product or relate to the discovery, manufacture, development or commercialization of a Product. The term PTC Patent Rights shall include PTC Base Patent Rights.

1.66 Quarterly R&D Fee

The term “Quarterly R&D Fee” shall mean the amount determined by (i) multiplying the FTE Rate by the number of FTEs contributed by PTC during the applicable Calendar Quarter or portion thereof as set forth in the then-current Research Plan, SRA Development Plan or New Product Development Plan, as applicable, it being understood that such amounts shall include, if not yet invoiced or paid, fees for PTC FTEs contributed prior to the applicable Calendar Quarter, and (ii) adding to the number in clause (i) all out-of-pocket costs incurred during the applicable Calendar Quarter as set forth in the then-current Research Plan, SRA Development Plan or New Product Development Plan, as applicable, it being understood that such amounts shall include, to the extent not yet invoiced or paid, any such amounts incurred by PTC prior to the applicable Calendar Quarter.

1.67 Regulatory Approval

The term “Regulatory Approval” shall mean any approvals (including without limitation pricing and reimbursement approvals), licenses, registrations or authorizations by a Regulatory Authority that are necessary for the manufacture and sale of a Product in the Field in a regulatory jurisdiction in the Territory.

1.68 Regulatory Authority

The term “Regulatory Authority” shall mean any national, supranational (e.g., the European Commission, the Council of the European Union, the European Medicines Agency), regional, state or local regulatory agency, department, bureau, commission, council or other governmental entity with responsibility for granting approvals, licenses, registrations, or authorizations necessary for the manufacturing and sale of pharmaceutical products in a country, including the FDA.

1.69 Research Plan

The term “Research Plan” shall mean the plan of research attached as Appendix 1.69 outlining the work expected to be performed by PTC and Roche hereunder, as such plan may be updated from time to time as provided in this Agreement. For clarity, as of the Effective Date and during the SRA Research Term, the Research Plan also constitutes the SRA Research Plan.

1.70 Research Program

The term “Research Program” shall mean the activities undertaken by the Parties pursuant to the Research Plan, including without limitation, such activities relating to SRA DC Research during the SRA Research Term, and New Product research during the Research Term.

1.71 Research Term

The term “Research Term” shall mean the period of time in which the Research Program shall be conducted, commencing on the Effective Date and continuing until terminated by Roche in accordance with Section 4.1.6.

1.72 Roche Background Patent Rights

The term “Roche Background Patent Rights” shall mean any and all Patent Rights, excluding the Roche Product Patent Rights and the excluded Patent Rights identified in Appendix 1.72, that are Controlled by Roche during the Agreement Term and either Cover a Product or relate to the discovery, manufacture, development or commercialization of a Product.

1.73 Roche Group

The term Roche Group shall mean collectively Roche, its Affiliates (including Chugai in the event Chugai is included as an Affiliate by Roche) and its Sublicensees.

1.74 Roche Know-How

The term “Roche Know-How” shall mean all Know-How that Roche Controls during the Agreement Term.

1.75 Roche Patent Rights

The term “Roche Patent Rights” shall mean the Roche Background Patent Rights and the Roche Product Patent Rights.

1.76 Roche Product Patent Rights

The term “Roche Product Patent Rights” shall mean any and all Patent Rights that are Controlled by Roche during the Agreement Term and that Cover the composition of matter or the method of use of a Product.

1.77 Royalty Term

The term “Royalty Term” shall mean, with respect to a Product and for a given country, the period of time commencing on the date of First Commercial Sale of the Product in such country and ending on the latest of the date that is (i) 10 (ten) years after the date of the First Commercial Sale of the Product in such country, (ii) the expiration of the last to expire PTC Patent Right, Roche Product Patent Right or Joint Patent Right in such country Covering the use, import, offering for sale, or sale of the Product in such country, or (iii) the duration of any applicable Marketing Exclusivity.

1.78 Sales

The term “Sales” shall mean, for a Product in a particular period, the amount stated in Roche “Sales” line of its externally published audited financial statements with respect to such Product for such period. This amount reflects the gross invoice price at which such Product was sold or otherwise disposed of (other than for use as clinical supplies or free samples) by Roche, its Affiliates and Sublicensees to Third Parties in such period reduced by gross-to-net deductions, if not previously deducted from such invoiced amount, taken in accordance with the then currently used IFRS.

By way of example, the gross-to-net deductions taken in accordance with IFRS as of the Effective Date include the following:

(a) credits, reserves or allowances granted for (i) damaged, outdated, returned, rejected, withdrawn or recalled Product, (ii) wastage replacement and short-shipments; (iii) billing errors and (iv) indigent patient and similar programs (*e.g.*, price capitation);

(b) governmental price reductions and government mandated rebates;

(c) chargebacks, including those granted to wholesalers, buying groups and retailers;

(d) customer rebates, including cash sales incentives for prompt payment, cash and volume discounts; and

(e) taxes, duties and any other governmental charges or levies imposed upon or measured by the import, export, use, manufacture or sale of a Product (excluding income or franchise taxes).

1.79 Second SRA Amendment

The term “Second SRA Amendment” shall mean Amendment No. 2 to the SRA, said Second SRA Amendment having an effective date of May 1, 2009.

1.80 SMA

The term “SMA” shall mean spinal muscular atrophy.

1.81 SRA

The term “SRA” shall mean the Sponsored Research Agreement by and between the Foundation and PTC, effective June 1, 2006, as amended by the First SRA Amendment, the Second SRA Amendment, the Third SRA Amendment and the Fourth SRA Amendment (each, an “Amendment”). A copy of the SRA, including all Amendments and appendices in existence as of the Execution Date, is attached as Appendix 1.81.

Each term listed under the “Definitions” column in the following table shall have the meaning ascribed pursuant to the SRA to the term listed in the corresponding row in the “Equivalent” column. For convenience, sections of the SRA and Second SRA Amendment that, as of the Execution Date, may be useful for interpreting the meaning of such Equivalent term pursuant to the SRA are set forth in the corresponding row in the columns thereafter. Such list of sections is not exhaustive and other sections of the SRA (including all amendments thereto) shall nevertheless be taken into account when interpreting the meaning of such Equivalent term.

		Sample sections useful for interpretation of Equivalent	
Definitions	**Equivalent**	**SRA**	**Second SRA Amendment**
SRA Buy-Out Notice	Buy-Out Notice		1(j), 8 (3.3(b)(v))
SRA Buy-Out Right	Buy-Out Right		1(j), 8 (3.3(b)(iv))
SRA Collaboration Activities	Collaboration Activities		1(j)
SRA Company Base IP	Company Base IP	1.4	
SRA Company Clinical Trial	Company Clinical Trial	1.5	

		Sample sections useful for interpretation of Equivalent	
Definitions	**Equivalent**	**SRA**	**Second SRA Amendment**
SRA Company Technology	Company Technology	1.8	
SRA Controlled	Control	1.10	
SRA Cost/Timeline Issue	Cost/Timeline Issue		2(g)
SRA Data	Data	1.11, 6.1(a)	1(b)
SRA DC Research	DC Research		1(j), 2(a)
SRA Drug Candidate	Drug Candidate	1.12	
SRA Development	Development		1(j)
SRA Development Candidate	Development Candidate		1(j)
SRA Development Deadline Document	Development Deadline Document		1(j), 8 (3.1)
SRA Development Plan	Development Plan		1(j)
SRA Field	Field	1.15	
SRA IND	IND	1.18	1(e)
SRA JSC	JSC		1(j), 5(a)
SRA Lead Candidate	Lead Candidate	1.21, 2.4(a)	
SRA NDA	NDA		1(j)
SRA Product	Product	1.27	
SRA Research	Research	1.30	
SRA Research Plan	Research Plan	1.34	2
SRA Research Term	Research Term	1.36, 2.6	2

SRA Reversion Candidate	Reversion Candidate		1(j), 5(b)(vi), 5(c)(i)
SRA Reversion Notice	Reversion Notice		1(j), 3(d), 8(a) (3.2, 3.4(a)(i), 3.4(b), 3.4(c), 3.4(d)), 12 (6.1(c)(1))
SRA Reversionary License	Reversionary License	1.38	1(h), 12 (6.1(c)(2)(i))
SRA Special Termination	Special Termination		1(j), 3

1.82 SRA Compound

The term "SRA Compound" shall mean any and all SRA Drug Candidates, SRA Reversion Candidates, SRA Development Candidates, and SRA Products. For clarity, SRA Compound includes without limitation the compounds referred to as [**]. In addition, SRA Compound shall include any small molecule compound first synthesized or identified hereunder by either Party (a) from and after the Effective Date and prior to [**] that, as a direct result of its therapeutic application, results in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA, unless PTC can establish through written documentation that such compound (i) [**] or [**] and (ii) [**], or (b) from and after [**] and that is [**] existing as of [**]. Each such additional SRA Compound shall be considered for the purposes of this Agreement to be an SRA Drug Candidate and for the purposes of the SRA to be a Drug Candidate, the SRA shall be deemed amended to the extent necessary to consider each such SRA Compound to be a Drug Candidate pursuant to the SRA, and such amendment shall survive expiration or termination of this Agreement.

1.83 Sublicensee

The term "Sublicensee" shall mean an entity to which Roche or its Affiliates have licensed rights pursuant to this Agreement.

1.84 Successful Completion of Pivotal Study

The term "Successful Completion of Pivotal Study" shall mean the results with respect to clinical endpoints and safety from a fully enrolled and completed (determined in accordance with the applicable protocol) Pivotal Study, as reflected in the final report from such study, support submitting an application for Regulatory Approval.

1.85 Territory

The term "Territory" shall mean all countries of the world.

1.86 Third SRA Amendment

The term "Third SRA Amendment" shall mean Amendment No. 3 to the SRA, said Third SRA Amendment having an effective date of January 1, 2011.

1.87 Third Party

The term "Third Party" shall mean a Person other than (i) PTC or any of its Affiliates, (ii) a member of the Roche Group, and (iii) solely with respect to the Foundation Provisions, the Foundation.

1.88 Transitional Research Plan

The term "Transitional Research Plan" shall mean the plan of research attached as Appendix 1.88 outlining the work expected to be performed by PTC and Roche hereunder between the Effective Date and the first meeting of the JSC. For clarity, the Transitional Research Plan also constitutes the SRA Research Plan during such time period.

1.89 US

The term "US" shall mean the United States of America and its territories and possessions.

1.90 US$

The term "US$" shall mean US dollars.

1.91 Valid Claim

The term "Valid Claim" shall mean, as applicable, any claim in any (i) unexpired and issued Patent Right that has not been disclaimed, revoked or held invalid by a final nonappealable decision of a court or other government agency of competent jurisdiction, or (ii) patent application that has not lapsed, in the case of a provisional patent application, or been cancelled, withdrawn or abandoned without the possibility of revival, nor has been pending for more than [**] years from the earliest priority date claimed for such application.

1.92 Additional Definitions

Each of the following definitions is set forth in the Section of this Agreement indicated below:

Definition	Section
Accounting Period	12.1
Acquired Party Activity	3.5(d)
Adjusted Gross Sales	1.50

Amendment	1.80
Bankruptcy Code	21
Breaching Party	20.3.1
Chairperson	7.2
Change of Control Notice	7.15
Chugai	1.1
Consensus Matter	7.7.3
CROs	5.1.4
Decision Period	15.5
Disclosing Party	1.14
DOJ	22.1
Election Notice	7.15
Enrollees	5.1.2

Definition	Section
Exclusivity Period	3.5(e)
Execution Date	Preface
FTC	22.1
H-W Suit Notice	15.11
Hub	22.4
Indemnified Party	17.3
Indemnifying Party	17.3
Infringement Notice	15.8
Initiating Party	15.8
Joint Invention	1.42
Joint IP Team or JIPT	7.4(m)
JOT	7.4(k)
Members	7.2
JSC	7.1
Non-Breaching Party	20.3.1
Patent Challenge	20.3.4
Patent Term Extensions	15.12
Patients	5.1.3
Payments	13
Peremptory Notice Period	20.3.1
Post-Change of Control Material Change	22.2
Pre-Approval Sales	11.5.6
PTC	Preface
PTC Invention	1.42
Publishing Notice	19.5
Publishing Party	19.5
R&D Event	11.3
R&D Event Payment	11.3
Receiving Party	1.14
Reverse Merger	1.9
Roche	Preface
Roche Basel	Preface
Roche Indemnitee	5.1.4
Roche Invention	1.42
Roche Losses	5.1.4
Roche Nutley	Preface
Settlement	15.8
SMAF Clinical Trials Advisory Committee	5.1.1
SPCs	15.12
SRA Licensee Data	2.2.2
SRA Licensee Technology	2.2.2
Suit Notice	15.8
Third Party Activity	3.5(c)

2. Foundation Obligations

2.1 General Rights and Obligations

Notwithstanding anything else in this Agreement, the rights and obligations of the Parties pursuant to this Agreement are subject to the terms of the SRA, except to the extent that such terms are expressly amended or replaced by this Agreement. By its acknowledgement and acceptance of this Agreement, Foundation agrees to be bound by the terms and conditions of the Foundation Provisions, and each Party shall have a right to enforce against Foundation any Foundation Provision pursuant to which Foundation has an obligation to such Party or pursuant to which such Party would benefit. Likewise, Foundation has a right to enforce, against a Party, any provision of this Agreement pursuant to which such Party has an obligation to Foundation (which obligations shall include obligations of PTC pursuant to the SRA that Roche is assuming pursuant to this Agreement; such assumption shall not be considered an amendment of the SRA and shall not limit Foundation's rights pursuant to the SRA) or pursuant to which Foundation would benefit. Foundation acknowledges and agrees that the JSC as described herein, once established and thereafter during the Agreement Term, shall replace in all respects the SRA JSC. Unless Roche provides its express written consent,

Roche shall not be bound to any modification or amendment made to the SRA after the Effective Date. Roche shall be a third party beneficiary under Article 1 (limited to the extent necessary to interpret the SRA) and Sections [**] of the SRA (including all amendments and attachments thereto).

2.2 Specific Rights and Obligations

In accordance with the Second SRA Amendment Section 10(d)(ii), Roche acknowledges and agrees to the following specific rights and obligations.

2.2.1 SRA Development of an SRA Development Candidate or SRA Product

To the extent Roche will assume responsibility for SRA Development of an SRA Development Candidate or SRA Product in any country, Roche assumes the obligations and rights of PTC pursuant to Second SRA Amendment Section 13 in that country, which such obligations and rights are set forth more specifically in 5.1.

2.2.2 Termination and Grant-back Provisions

Roche's rights and licenses from PTC with respect to SRA Reversion Candidates, SRA Development Candidates and SRA Products will terminate upon an SRA Special Termination or PTC's receipt of an SRA Reversion Notice or SRA Buy-Out Notice and Roche shall be obliged in such circumstances to grant the licenses and rights specified in Section 3 of the Second SRA Amendment and Section 6.1(c)(2) of the SRA (including licenses and rights to (A) all intellectual property that, if developed, acquired or otherwise SRA Controlled by PTC, rather than a member of the Roche Group, would be SRA Company Technology or SRA Data ("**SRA Licensee Technology**" and "**SRA Licensee Data**", respectively) and (B) all SRA INDs, SRA NDAs or similar regulatory filings made or obtained by a member of the Roche Group with respect to the relevant SRA Reversion Candidates, SRA Development Candidates and SRA

Products) and perform the activities specified therein in each case as if Roche were PTC. PTC shall use Commercially Reasonable Efforts to keep the SRA in full force and effect and not trigger an SRA Special Termination, SRA Reversion Notice or SRA Buy-Out Notice. For clarity, nothing in this Section 2.2.2 shall be interpreted as limiting PTC's obligations pursuant to the SRA, including its obligations pursuant to Sections 3.2 and 6.1(c)(2) of the SRA.

2.3 Designation of SRA Development Candidate

The Parties and the Foundation shall use good faith, diligent efforts to designate an SRA Development Candidate as soon as possible after the Effective Date and in any event prior to [**]. Each SRA Compound that progresses to IND-enabling pre-clinical studies in support of future human clinical trials, regardless of the date of such progression, shall be considered for the purposes of this Agreement to be an SRA Development Candidate and for the purposes of the SRA to be a Development Candidate. The SRA shall be deemed amended to the extent necessary to consider such SRA Compound to be a Development Candidate pursuant to the SRA, and such amendment shall survive expiration or termination of this Agreement.

3. Grant of Licenses

3.1 License to Roche

Subject to Section 2.2.2, Section 3.2 and the other terms and conditions of this Agreement, PTC hereby grants to Roche and its Affiliates a royalty-bearing, exclusive (even as to PTC, but subject to the retained right of PTC to perform its obligations under the Research Program and its obligations (if any) under the Product Development Program) right and license, including the right to grant sublicenses in conformance with Section 3.3, under PTC's interest in the PTC Patent Rights and PTC Know-How and PTC's interest in the Joint Patent Rights and Joint Know-How to research, have researched, develop, have developed, register, have registered, use, have used, make, have made, import, have imported, export, have exported, market, have marketed, distribute, have distributed, sell and have sold Compounds and Products in the Field in the Territory.

3.2 License to PTC

Roche hereby grants to PTC a non-exclusive, worldwide, paid-up right and license, without the right to sublicense, under the Roche Patent Rights and Roche Know-How and Roche's interest in the Joint Patent Rights and Joint Know-How solely to enable PTC to perform its obligations under the Research Program during the Research Term and its obligations (if any) under the Product Development Program.

3.3 Sublicense

Roche and its Affiliates shall have the right to sublicense or subcontract to its Affiliates (with the right to grant sublicenses in conformance with this Section 3.3) and to any other Person (with no further right to sublicense), and shall have the right to use any contract manufacturer, distributor, subcontractor or outsourced service for the benefit of Roche and its Affiliates. For clarity, Roche need not obtain the permission of PTC in order to enter into a sublicense or subcontract. Roche shall provide PTC with written notice of any such sublicense within [**]

days after the execution thereof, such written notice to include (i) the identity of the Sublicensee, (ii) the Compound(s) and Product(s) that are the subject of the sublicense, (iii) whether such sublicense is exclusive, co-exclusive or non-exclusive, and (iv) the territory(-ies) covered by such sublicense. If Roche grants a sublicense, all of the terms and conditions of this Agreement shall apply to the Sublicensee to the same extent as they apply to Roche for all purposes of this Agreement, and the sublicense shall include provisions causing such sublicense to terminate upon an SRA Special Termination or PTC's receipt of notice of any SRA Reversion Notice or SRA Buy-Out Notice and requiring such Sublicensee to provide Roche with all rights necessary or useful for Roche to comply with Section 2.2.2; Foundation shall be a third party beneficiary of such provisions. Roche assumes full responsibility for the performance of all obligations so imposed on such Sublicensee and will itself pay and account to PTC for all payments due under this Agreement by reason of operation of any such sublicense.

3.4 Rights Retained by the Parties

Any rights of PTC or Roche, as the case may be, not expressly granted to the other Party pursuant to this Agreement shall be retained by such Party. Without limiting the generality of the foregoing, no right or license is granted to Roche under the PTC Patent Rights and PTC Know-How and PTC's interest in the Joint Patent Rights and Joint Know-How to research, have researched, develop, have developed, register, have registered, use, have used, make, have made, import, have imported, export, have exported, market, have marketed, distribute, have distributed, sell and have sold any composition that is not a Compound or Product. Except in the context of a mutually agreed Research Plan, the exclusive license granted hereunder excludes any right of Roche or its Affiliates to access or use the AS Assay or to grant any such rights to the AS Assay to any other Person.

3.5 Exclusivity.

(a) During the Exclusivity Period (as defined in Section 3.5(e)), both Parties shall collaborate exclusively with each other pursuant to this Agreement with regard to use of Alternative Splicing to identify small molecule compounds that, as a direct result of their therapeutic application, result in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA, and neither Party nor its Affiliates shall, except pursuant to this Agreement, either alone or in collaboration with a Third Party (a) engage in discovering, researching, developing, registering, having registered, using, having used, making, having made, importing, having imported, exporting, having exported, marketing, having marketed, distributing, having distributed, selling and having sold any compound (or any product containing a compound) in the Field, which compound or product such Party or its Affiliates knows or believes to be a small molecule compound identified with Alternative Splicing that, as a direct result of its therapeutic application, results in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA, or (b) grant a license to, or otherwise assist or contract with, any Third Party, to engage in discovering, researching, developing, registering, having registered, using, having used, making, having made,

importing, having imported, exporting, having exported, marketing, having marketed, distributing, having distributed, selling and having sold any compound (or any product containing a compound) in the Field, which compound or product such Party or its Affiliates knows or believes to be a small molecule compound identified with Alternative Splicing that, as a direct result of its therapeutic application, results in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA.

(b) For purposes of clarity, the restrictions set forth in Section 3.5(a) shall not prohibit either Party from using Alternative Splicing to identify small molecule compounds that result in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA, so long as the primary goal of such program, as evidenced by laboratory notebooks or other relevant documents, is not to discover or develop compounds that, as a direct result of their therapeutic application, result in increased levels of SMN1 mRNA and protein based on the conversion of SMN2 RNA to SMN1 mRNA.

(c) This Section 3.5 is not intended to apply to any activity otherwise prohibited by this Section 3.5 if a Party's involvement in such prohibited activity results from such Party's acquisition by a Third Party (either directly or through any Affiliate, whether by merger, purchase of assets or equity, or otherwise), but only if (i) such Third Party, prior to such acquisition or merger, was already engaged in such prohibited activity (the "**Third Party Activity**"), and (ii) no Patent Rights or Know-How of either Party, nor any Joint Patent Rights or Joint Know-How, are used in connection with such Third Party Activities.

(d) This Section 3.5 is not intended to apply to any activity otherwise prohibited by this Section 3.5 if a Party's involvement in such prohibited activity results from such Party's acquisition (either directly or through any Affiliate, whether by merger, purchase of assets or equity, or otherwise) of the whole or substantially the whole of the business or assets of a Third Party, but only if (i) such Third Party, prior to such acquisition or merger, was already engaged in such prohibited activity (the "**Acquired Party Activity**"), and (ii) such acquiring Party shall, within [**] days after the date of such Party's consummation of such acquisition, notify the other Party of such acquisition and comply with the other requirements of this Section 3.5(d). After consummation of such an acquisition, the acquiring Party shall, at its option, either (x) use good faith efforts to identify a Third Party purchaser to whom such Party will divest its interest in the Acquired Party Activity and to enter into a definitive agreement with such Third Party for such divestiture as soon as reasonably practicable under the circumstances, but such divestiture must be completed no later than [**] months after the closing of such Party's acquisition of the Acquired Party Activity, or (y) promptly discontinue such Acquired Party Activity;

provided that notwithstanding which option is elected, such divesture or discontinuation must be accomplished no later than [**] months after the closing of such Party's acquisition of the Acquired Party Activity. During the time period after consummation of an acquisition covered by this Section 3.5(d) and until the divestiture or discontinuation of the Acquired Party Activity, the acquiring Party shall not use any Patent Rights or Know-How of either Party, or any Joint Patent Rights or Joint Know-How, in connection with such Acquired Party Activities. So long as the acquiring Party divests of, or discontinues, the Acquired Party Activity in accordance with this Section 3.5(d), such acquisition shall not be deemed a violation of this Section 3.5.

(e) As used in this Agreement, the term "**Exclusivity Period**" means the period commencing on the first day of the Research Term and ending three (3) years thereafter.

4. Research Collaboration

4.1 Conduct of Research Program

4.1.1 Scope

Roche and PTC shall conduct research activities under this Agreement under the oversight of the JSC and a mutually agreed Research Program pursuant to the Research Plan.

4.1.2 Diligent Efforts

Roche and PTC shall each use Commercially Reasonable Efforts to perform their respective tasks and obligations in conducting all activities ascribed to it in the then-current Research Plan, in accordance with the time parameters set forth therein.

4.1.3 Transitional Research Plan

PTC will conduct the Research Program in accordance with the Transitional Research Plan until the time of the first JSC meeting; provided, however, that PTC shall have the right, but not the obligation, at its cost, to apply resources to the Research Program during the period of the Transitional Research Plan as long as such resources are applied in a manner consistent with the Research Plan.

4.1.4 Research Plan

After the first JSC meeting, the Parties will conduct the Research Program in accordance with the Research Plan; provided, however, that PTC shall have, if permitted by the JSC, the right, but not the obligation, at its cost, to apply resources to the Research Program after the Transitional Research Plan as long as such resources are applied in a manner consistent with the Research Plan.. Prior to the expiration of the SRA Research Term, any changes to the Research Plan shall be considered amendments to the SRA Research Plan following approval by the JSC. Following the SRA Research Term, unless decided otherwise by the JSC, the Research

Plan will be updated annually by the JSC. The Research Plan will set forth (i) the scope of the Research Program and the resources that will be dedicated to the activities contemplated within the scope of the Research Program, including the responsibilities of each Party (and, if as applicable, the Foundation), (ii) specific objectives for each year, which objectives will be updated or amended by the JSC as research progresses, and (iii) budgets for such activities. The Parties shall prepare a plan for activities to be conducted during the second year of the Agreement Term no later than [**] days before the first anniversary of the Effective Date. Any changes to the Research Plan shall be reflected in written amendments to the Research Plan.

4.1.5 Duration

The Research Program shall commence on the Effective Date and shall continue until the end of the Research Term.

4.1.6 Termination

The Research Term may be terminated by Roche at any time following the second anniversary of the Effective Date upon ninety (90) days advance written notice to PTC, which notice can be provided in advance of the second anniversary of the Effective Date.

4.1.7 Expenses

Roche shall bear all expenses of the Research Program, including without limitation the payment to PTC of Quarterly R&D Fees as provided in Section 11.2.

4.2 Records; Reports

4.2.1 Progress Reports

At least quarterly during the Research Term, any Party actively conducting work under the Research Program shall have the obligation to prepare and provide to the JSC a detailed written report summarizing the progress of the work performed by that Party in the course of the Research Program during the preceding Calendar Quarter, if any. Promptly upon expiry of the Research Term, each Party shall provide a final written report summarizing its activities under the Research Program and the results thereof. Each Party shall promptly provide Foundation with a copy of each report prepared by such Party pursuant to this Section 4.2.1. Upon the written request of Roche and not more than [**], PTC shall permit Roche, at Roche's expense, to have access during PTC's Business Days to those records of PTC that may be necessary to verify the basis for any payments hereunder.

4.2.2 Research Records

Each Party shall maintain records of the Research Program (or cause such records to be maintained), which shall be completely and accurately recorded in separate laboratory notebooks, in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes.

5. Product Development

5.1 SRA Product Development

Roche, at its sole cost, shall be responsible for pursuing clinical development of SRA Products, including any non-clinical studies required to support such clinical development, subject to the terms of the SRA (with Roche having responsibility for fulfilling all of PTC's obligations thereunder with respect to clinical development of SRA Products).

5.1.1 SMAF Clinical Trials Advisory Committee

Foundation has the right, but not the obligation, to create a committee of experts to advise Foundation/Roche/PTC, as applicable, on clinical trials and expanded access with respect to SRA Development Candidates and SRA Products (the "**SMAF Clinical Trials Advisory Committee**"). Such SMAF Clinical Trials Advisory Committee shall consist of such individuals as Foundation may designate, but shall include [**]. The SMAF Clinical Trials Advisory Committee shall have, as one (1) of its principal mandates, the responsibility of balancing (i) the rapid and efficient development and commercialization of SRA Development Candidates and SRA Products for the benefit of all potential patients in the SRA Field and (ii) the appropriateness, based on available safety and efficacy information with respect to such SRA Development Candidates and SRA Products, of providing access to such SRA Development Candidates and SRA

Products to individual patients via the extension protocols or expanded access programs further described in Sections 5.1.2 and 5.1.3. Such SMAF Clinical Trials Advisory Committee may establish its own procedures for meetings and decision-making.

5.1.2 Roche Clinical Trials

Foundation has the right, but not the obligation, to assist with patient recruitment for any SRA Company Clinical Trial involving SMA patients by (i) referring to Roche (or, at Roche's request, referring directly to any clinical investigator at a clinical trial site for the applicable clinical trial) up to [**] SMA patients meeting the enrollment criteria for the applicable clinical trial and identified by Foundation or its designee, and/or (ii) proposing up to [**] clinical trial sites with access to appropriate patient populations for such clinical trial. With regard to clinical trials involving any Compound or Product, Roche shall use Commercially Reasonable Efforts to enable such patients to be enrolled in such clinical trial consistent with the applicable enrollment criteria, protocol, and target patient number for such clinical trial (it being understood that such patients should be given priority over other patients who are equally qualified to participate in such clinical trial, provided that the final decision regarding such enrollment is made by the clinical investigator and/or clinical trial site personnel of the investigating institution), and to contract with such clinical trial sites for such clinical trial. If Foundation, in its sole discretion, determines not to assist in patient recruitment for any such clinical trial, then it shall so inform Roche and Roche shall assume all responsibility for patient recruitment and selection of clinical trial sites.

Each time that Roche commences the drafting of a clinical trial protocol for a SRA Development Candidate or SRA Product, and at reasonable times thereafter, Roche will discuss with Foundation Roche's plans for making such SRA Development Candidate or SRA

Product available to participants in such clinical trial after the completion of such trial. If mutually agreed by Roche and Foundation based on such discussions, or if recommended by the SMAF Clinical Trials Advisory Committee in its sole discretion, Roche will submit to the appropriate Regulatory Authority a suitable extension protocol and corresponding informed consent form providing for administration of such SRA Drug Candidate or SRA Product for at least [**] beyond the term provided for in a particular clinical trial. Roche shall use Commercially Reasonable Efforts to obtain the applicable Regulatory Authority's approval of such extension protocol and informed consent and subsequent approval from the institutional review boards at the locations where such clinical trial is being conducted; provided, however, that the proposed [**] period for such extension protocol may be shortened based on the request or advice of the applicable Regulatory Authority. Upon receipt of such approvals, Roche shall provide, in accordance with the approved extension protocol, such SRA Development Candidate or SRA Product to those patients who enrolled in such clinical trial (such patients, the "**Enrollees**"). For so long as Roche is continuing to develop or seek approval from a Regulatory Authority for such SRA Development Candidate or SRA Product, and subject either to mutual agreement of Roche and Foundation or to the recommendation of the SMAF Clinical Trials Advisory Committee in its sole discretion, Roche shall use Commercially Reasonably Efforts to obtain approval for an amended or new extension protocol providing for continued administration of such SRA Development Candidate or SRA Product to the Enrollees, and Roche shall provide such SRA Development Candidate or SRA Product to the Enrollees in accordance with any such approved protocol. If Roche [**] does not concur in the decision to commence or continue any extension protocol pursuant to this Section 5.1.2, then Roche's obligations to assist with such extension protocol and continue to supply such SRA Development Candidate or SRA Product to Enrollees shall [**] directly or indirectly, [**], and [**] of SRA Development Candidate or SRA Product to Enrollees.

If Roche stops developing or seeking approval from a Regulatory Authority of a SRA Development Candidate or SRA Product pursuant to the above paragraph, and either Roche and Foundation mutually agree or the SMAF Clinical Trials Advisory Committee in its sole discretion (but having considered any safety issues) recommends that the Enrollees continue to have access to such SRA Development Candidate or SRA Product for a longer period than provided for in any existing extension protocol submitted by Roche with respect to such SRA Development Candidate or SRA Product, then upon the Foundation's request, Roche shall facilitate Foundation's efforts to arrange for prolonged continued access to such SRA Development Candidate or SRA Product for some or all of the Enrollees by taking all reasonable actions requested by Foundation (consistent with the SMAF Clinical Trials Advisory Committee's recommendations, if applicable), including without limitation: (i) either (1) transferring Roche's SRA IND for such SRA Development Candidate or SRA Product to Foundation or its designee or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Roche's SRA IND or Drug Master File or equivalent regulatory filing (as applicable) so that Foundation or its designee can submit its own SRA IND with respect to such continued access; (ii) providing (for the shorter of [**] months or the amount of time necessary for Foundation or its designee to establish an alternative supply of equivalent clinical grade product) such SRA Development Candidate or SRA Product to Foundation or its designee for administration to such Enrollees in accordance

with any extension protocol for which Foundation or its designee has obtained approval from the FDA or the applicable Regulatory Authority; (iii) assisting Foundation or its designee with obtaining an alternative, equivalent clinical grade supply of such SRA Development Candidate or SRA Product by (1) facilitating Foundation's or its designee's negotiation of a supply agreement with Roche or Roche's manufacturer of such SRA Development Candidate or SRA Product or (2) providing technology transfer and other technical assistance reasonably requested by Foundation to enable Foundation or its designee to manufacture such SRA Development Candidate or SRA Product; and (iv) providing Foundation with a non-exclusive, fully paid-up, sublicensable license under SRA Licensee Technology and SRA Licensee Data (provided, that the license granted hereunder to Foundation [**] or [**] or [**]) to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide continued access to such SRA Development Candidate or SRA Product in accordance with this Section 5.1.2. In any case in which Roche [**], does not concur in the decision to commence or continue any extension protocol pursuant to this Section 5.1.2, then Roche's obligations to assist with such extension protocol and continue to supply such SRA Development Candidate or SRA Product to Enrollees shall [**], directly or indirectly, [**], and [**] of SRA Development Candidate or SRA Product to Enrollees. In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to losses arising out of or related to the activities contemplated under this Section 5.1.2. Foundation shall provide a certificate of insurance evidencing such coverage to Roche upon request.

For clarity, nothing in this Section 5.1.2 shall be interpreted as limiting PTC's obligations pursuant to the SRA, including to provide licenses to SRA Company Technology, SRA Data and SRA Company Base IP to facilitate Foundation's efforts to arrange for prolonged continued access to such SRA Development Candidate or SRA Product for some or all of the Enrollees.

5.1.3 Expanded Access Program

At such time as Foundation and Roche mutually agree, or the SMAF Clinical Trials Advisory Committee in its sole discretion determines, that results from clinical trials and other development activities with respect to the applicable SRA Development Candidate or SRA Product support expanded access

to such SRA Development Candidate or SRA Product for patients with SMA, then Roche and Foundation shall cooperate to establish such an expanded access program in which at least [**] SMA patients identified by Foundation who do not meet the enrollment criteria for a particular clinical trial (whether or not such clinical trial is directed to SMA patients) for such SRA Development Candidate or SRA Product (such patients, the "**Patients**") may gain access to such SRA Development Candidate or SRA Product. Roche agrees that at its earliest reasonable opportunity following the commencement of such cooperation (e.g., at a meeting with the FDA), Roche will inquire about the feasibility of an expanded access protocol for such SRA Development Candidate or SRA Product for SMA

purposes and will invite a designee of Foundation with appropriate medical or regulatory experience to participate in discussions with the FDA regarding the establishment and maintenance of such expanded access program. In connection with such expanded access program, at Foundation's request and consistent with any recommendations made by the SMAF Clinical Trials Advisory Committee, Roche will either (i) submit to the FDA a protocol that is reasonably acceptable to Foundation and calls for administering such SRA Development Candidate or SRA Product to the Patients or (ii) notify Foundation that it will not be making such a submission and facilitate the submission and approval of such a protocol by the Foundation or its designee.

If Roche chooses option (i) above, then it shall use Commercially Reasonable Efforts to obtain approval of such protocol and, upon receipt of such approval, it shall provide such SRA Development Candidate or SRA Product to the Patients in accordance with the approved protocol; provided, that Roche and Foundation shall engage in good faith negotiations with respect to [**].

If Roche chooses option (ii) above, then Roche shall facilitate Foundation's efforts to arrange for such expanded access program for such SRA Development Candidate or SRA Product for the Patients by taking all reasonable actions requested by Foundation, in each case [**], including without limitation: either (1) allowing the expanded access program to be performed pursuant to Roche's SRA IND (in which case Foundation or its designee shall provide Roche with all data arising from and other information with respect to such expanded access program that is necessary or reasonably useful for Roche to fulfill its obligations as the SRA IND holder) or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Roche's SRA IND or Drug Master File or similar regulatory filing (as applicable) so that Foundation or its designee can file its own SRA IND with respect to such expanded access program; (ii) providing such SRA Development Candidate or SRA Product to an appropriate designee of Foundation for administration to the Patients in accordance with any expanded access protocol for which Foundation or its designee has obtained approval from the FDA [**]; and (iii) providing Foundation with a non-exclusive, fully paid-up, sublicensable license under SRA Licensee Technology and SRA Licensee Data (provided, that the license granted hereunder to Foundation [**] or [**] or [**]) to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide such expanded access to such SRA Development Candidate or SRA Product in accordance with this Section 5.1.3. In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with respect to losses arising out of or related to the activities contemplated under this Section 5.1.3. Foundation shall provide a certificate of insurance evidencing such coverage to Roche upon request.

For clarity, nothing in this Section 5.1.3 shall be interpreted as limiting PTC's obligations pursuant to the SRA, including to provide licenses to SRA Company Technology, SRA Data and SRA Company Base IP to facilitate Foundation's efforts to arrange for such

expanded access program for such SRA Development Candidate or SRA Product for the Patients.

5.1.4 Indemnification by Foundation

In connection with the last paragraph in each of Section 5.1.2 and Section 5.1.3, Foundation hereby agrees to save, defend, indemnify, and hold harmless Roche's and its Affiliates' trustees, officers, employees and agents (each, a "**Roche Indemnitee**") from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expenses and attorneys' fees ("**Roche Losses**"), to which a Roche Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Roche Losses arise directly or indirectly out of (a) [**] or [**] of any SRA Development Candidate or SRA Product by Foundation, its Affiliate(s) or licensee(s) pursuant to the last paragraph of Section 5.1.2 or 5.1.3, or (b) the breach of the SRA by Foundation or the gross negligence or willful misconduct of Foundation pursuant to the last paragraph of Section 5.1.2 or 5.1.3, except in each case to the extent such Losses result from (x) the breach of this Agreement by Roche or the gross negligence or willful misconduct of any Roche Indemnitee, or (y) the activities of Roche or its agents or employees in connection with any SRA Development Candidate or SRA Product. The obligations of Foundation under this Section 5.1.4 are conditioned upon Roche's delivery of written notice to Foundation of any potential Roche Losses promptly after Roche becomes aware of such potential Roche Losses. Foundation shall have the right to assume the defense of any suit or claim related to Roche Losses if it has assumed responsibility for the suit or claim in writing. If Foundation defends the suit or claim, Roche may participate in (but not control) the defense thereof at its sole cost and expense but Roche may not settle such suit or claim without the prior written consent of Foundation, not to be unreasonably withheld.

5.1.5 Clinical Trial/CRO Agreements

In connection with the last paragraph in each of Section 5.1.2 and Section 5.1.3, Foundation hereby agrees that under any circumstance in which Foundation is contracting directly with clinical trial sites, clinical investigators, and contract research organizations ("**CROs**"), it will use as the basis for its negotiations [**], and will use Commercially Reasonable Efforts to secure terms with respect to publication, confidentiality, intellectual property (which shall be [**], as the case may be, [**]), and indemnification substantially similar to those routinely obtained by Roche with respect to such an agreement, and naming Roche as a third-party beneficiary.

5.2 New Product Development

Roche, at its sole cost, shall be responsible for pursuing clinical development of New Products in accordance with a New Product Development Plan, and as overseen by the JSC. At Roche's option, PTC may assist with mutually agreed clinical and non-clinical activities in accordance with the New Product Development Plan, which shall include, without limitation, mutually agreed budgets for such activities. At Roche's reasonable request and at the sole expense of Roche (including for out-of-pocket and internal costs (including a reasonable allocation of overhead) incurred by the Foundation in connection therewith), the Foundation

shall use good faith efforts to assist with recruitment of SMA patients for Clinical Studies conducted in the United States under the New Product Development Plan.

6. Diligence

Roche and PTC shall use Commercially Reasonable Efforts to perform their respective activities contemplated by this Agreement or as may be agreed upon in any subsequent written agreements with respect to the subject matter hereof, including but not limited to any activities under the Research Program and the Product Development Program. Specifically, Roche agrees to use Commercially Reasonable Efforts to develop and commercialize Products in the Field as it relates to the treatment of humans having SMA and in the Territory and agrees to fulfill all diligence obligations imposed on PTC under the SRA. Roche further agrees to fulfill the same diligence obligations with respect to New Compound Development Candidates and New Products as are required under the SRA with respect to SRA Development Candidates and SRA Products, respectively.

7. Governance

7.1 Joint Steering Committee

Within [**] days after the Effective Date of this Agreement, the Parties and the Foundation shall establish a joint steering committee ("**JSC**") to oversee the Research Program and the Product Development Program activities under this Agreement.

7.2 Acknowledgement Concerning the JSC

7.2.1 Sole Governance Body for Certain Decisions

Each Party and the Foundation acknowledges that the JSC, in addition to its other functions as described herein, shall be the sole governance body for all research and SRA Development decisions regarding the SRA DC Research and the SRA Development of SRA Reversion Candidates, SRA Development Candidates and SRA Products that are the subject of this Agreement.

7.2.2 Excepted Decisions

The JSC's role as such sole governance body under Section 7.2.1 shall not prevent PTC or Roche from making decisions necessary or useful to implement decisions made by the JSC regarding research, SRA Development and SRA Collaboration Activities with respect to SRA Compounds, so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision.

7.3 Members

The JSC shall be composed of [**] persons ("**Members**"). Roche, PTC and the Foundation each shall be entitled to appoint [**] Members with appropriate seniority and functional expertise. Each Member shall be an employee of the appointing Party or the Foundation, as applicable, unless otherwise agreed by the other Party and the Foundation, or the

Parties, as applicable. Each Party and the Foundation may replace any of its Members and appoint a person to fill the vacancy arising from each such replacement. If PTC, Roche or the Foundation replaces a Member, it shall notify the other Party and the Foundation, or the Parties, as applicable, at least [**] days prior to the next scheduled meeting of the JSC. Both Parties and the Foundation shall use Commercially Reasonable Efforts to keep an appropriate level of continuity in representation. Members may be represented at any meeting by another person designated by the absent Member. The JSC shall be chaired by a Roche Member (the "**Chairperson**"). The Chairperson shall be responsible for calling meetings of the JSC and for leading the meetings.

7.4 Responsibilities of the JSC

The JSC shall have the responsibility and authority to:

a) monitor and implement the transfer of the SRA Product to Roche;

b) oversee, monitor and approve the Research Plan;

c) revise and approve any revisions to the Research Plan;

d) review and oversee the execution of the Research Plan;

e) establish timelines and criteria for decision points;

f) review the efforts of the Parties and allocate those resources for the Research Plan (including the budget);

g) identify appropriate resources necessary to conduct the Research Plan;

h) decide whether to pursue options (1), (2) or (3) of Section 2(g) of the Second SRA Amendment in the event of an SRA Cost/Timeline Issue;

i) maintain and update, at each JSC meeting prior to [**], a list that identifies and rank orders all potential and actual SRA Lead Candidates and SRA Development Candidates and denotes all SRA Development Candidates and between [**] potential or actual SRA Lead Candidates as SRA Reversion Candidates and as "Reversion Candidates" under the SRA;

j) prior to the designation of an SRA Development Candidate, prepare the SRA Development Plan and the "Development Plan" under the SRA for such SRA Development Candidate, and review and update the SRA Development Deadline Document and the "Development Deadline Document" under the SRA as it may deem advisable, in each case as further provided in Article 3 of the SRA;

k) establish and set expectations and mandates for each joint operational team (each, a "**JOT**");

l) create or disband JOTs as deemed appropriate;

m) oversee the JOTs, including without limitation the joint intellectual property team ("**Joint IP Team**" or "**JIPT**");

n) approve variations of Invention assignment agreements pursuant to Section 15.1;

o) revise and approve any revisions to the Development Candidate Criteria;

p) determine whether an SRA Drug Candidate has become an SRA Development Candidate or whether New Compound has become a New Compound Development Candidate;

q) following the designation of an SRA Development Candidate, and at least [**] thereafter, conduct a formal review and comprehensive update of the SRA Development Plan and SRA Development Deadline Document for such SRA Development Candidate, in each case as further provided in Article 3 of the SRA;

r) review scientific and medical literature to identify diseases, indications or medical conditions that, [**] are [**] for [**], and [**] and [**] diseases, indications or medical conditions [**];

s) at the first JSC meeting after [**], (i) review each potential or actual SRA Lead Candidate that was not designated as an SRA Development Candidate during the SRA Research Term and either designating it as an SRA Development Candidate or determining that it does not meet the Development Candidate Criteria, and (ii) prepare a final list (which can only be subsequently changed by the written agreement of Roche, PTC and Foundation) that identifies and rank orders all potential and actual SRA Lead Candidates and SRA Development Candidates and denotes all SRA Development Candidates and between [**] potential or actual SRA Lead Candidates as SRA Reversion Candidates and as "Reversion Candidates" under the SRA;

t) oversee the progress of the Product Development Program, including without limitation, with respect to SRA Development, pursuant to Article 3 of the SRA;

u) monitor the development of the New Compounds and the New Products in the Field;

v) recommend action items to each Party's or the Foundation's respective decision-making bodies for approval or rejection by such decision-making bodies;

w) attempt to resolve any disputes on an informal basis; and

x) otherwise serve as a forum for exchanging information and discussing the progress of the collaboration between PTC, Roche and the Foundation under this Agreement, including, without limitation, Product commercialization activities.

The JSC shall have no responsibility and authority other than that expressly set forth in this Section.

7.5 Meetings

The Chairperson or his/her delegate is responsible for sending invitations and agendas for all JSC meetings to all Members at least [**] days before the next scheduled meeting of the JSC, and shall include any agenda item reasonably requested by PTC or the Foundation. The venue for the meetings shall be agreed by the JSC. During the Research Term, the JSC shall meet at least [**]; thereafter, the JSC shall meet at least [**] per Calendar Year. JSC meetings may be held in person or by videoconference; provided that no more than [**] such meetings in any Calendar Year may be by videoconference. The Alliance Directors may attend the JSC meetings as permanent participants.

At least [**] representatives from each Party and from the Foundation must be present at each JSC meeting to achieve a quorum. In addition, each Party and the Foundation may, at its discretion, invite a reasonable number of non-voting employees, and, with the consent of the other Party and the Foundation, or the Parties, as applicable, consultants or scientific advisors, to attend JSC meetings or the relevant portion thereof; provided that any such consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement.

Either Party or the Foundation may also request that a special JSC meeting be convened for the purpose of resolving a dispute(s) in connection with, or for the purpose of reviewing or making a decision pertaining to, any matter within the purview of the JSC by providing written notice to the other Party. Such meeting shall be convened at such time as may be mutually agreed upon by the Parties, but in any event shall be held within [**] days after the date of such notice.

At its initial meeting, the JSC shall, among other things, review the Research Plan, including the associated budgets.

7.6 Minutes

The Chairperson is responsible for designating a Member to record in reasonable detail and circulate draft minutes of JSC meetings to all members of the JSC for comment and review within [**] days after the relevant meeting. The Members of the JSC shall have [**] days to provide comments. The Member preparing the minutes shall incorporate timely received comments and distribute finalized minutes to all Members of the JSC within [**] days after the relevant meeting, for final approval at the next JSC meeting.

7.7 Decisions

7.7.1 Decision Making Authority

The JSC shall decide matters within its responsibilities as set forth in Section 7.4.

7.7.2 Consensus; Good Faith

The Members of the JSC shall act in good faith to cooperate with one another and seek agreement with respect to issues to be decided by the JSC. The Parties and the Foundation shall endeavor to make decisions by consensus. Each Party and the Foundation shall have one (1) vote on the JSC; provided, however that the Foundation [**] except to the extent the Foundation [**] under the [**] (and, for clarity, Foundation shall not be obligated to [**] without Foundation's prior approval). Both Parties, and, as applicable, the Foundation, must vote in the affirmative to allow the JSC to take any action that requires the vote of the JSC. Action on any matter may be taken at a meeting by teleconference, videoconference or by written agreement.

7.7.3 Escalation

If the JSC is unable to decide a matter, then such matter shall be referred to the Chief Executive Officer of PTC or equivalent position or his/her nominee, the Head of Roche Partnering or equivalent position or his/her nominee, and, for matters on which the Foundation is entitled to vote, the President of the Foundation or equivalent position or his/her nominee, for resolution, who together shall use reasonable and good faith efforts to reach a decision by consensus within [**] days after the date such matter is referred to them. If PTC, Roche and the Foundation still fail to reach a decision within such [**] days, then the final decision shall be Roche's, which shall be exercised in good faith. Any such decision shall constitute a decision of the JSC.

Notwithstanding the foregoing, Roche shall not have the right to exercise its final decision-making authority unilaterally with respect to the following (each, a "**Consensus Matter**"):

a) increasing PTC's or the Foundation's obligations or reduce PTC's or the Foundation's rights under this Agreement, including without limitation, amending the Research Plan and/or imposing any obligation on PTC and/or the Foundation to devote additional personnel or financial resources to a specific activity or project;

b) determining that the R&D Events set forth in Section 11.3 have not occurred;

c) determining that it has fulfilled any obligation under this Agreement or that PTC or the Foundation has breached any obligation under this Agreement;

d) making a decision that is expressly stated in this Section 7.7.2 or elsewhere in this Agreement to require the mutual agreement of the Parties or the Foundation;

e) otherwise expanding Roche's rights or reducing Roche's obligations under this Agreement;

f) designating, or determining not to designate, an SRA Development Candidate;

g) updating or amending any SRA Development Plan or SRA Development Deadline Document; or

h) deciding whether to pursue option (1), (2) or (3) of Section 2(g) of the Second SRA Amendment.

7.8 Information Exchange

Roche shall disclose to PTC and the Foundation all material information in relation to Roche's activities under this Agreement through the JSC and, where applicable, the JSC, the Foundation and PTC may ask reasonable questions in relation to such matters and offer advice in relation thereto, and Roche shall give due consideration to PTC's and the Foundation's input.

7.9 Joint Operational Teams

7.9.1 Establishment

The JSC shall have the right to establish JOTs, which may include, but will not be limited to the following: a Research JOT, a New Product Development JOT, an SRA Development JOT, and a Joint IP Team. Each Party and the Foundation shall be free to change its members of each JOT, on prior written notice to the other Party and the Foundation. Each JOT shall determine the frequency and form of its interactions, subject to the requirements set forth in this Section 7.9.

7.9.2 Role of JOTs

The JSC shall determine the objectives and areas of responsibility for each JOT, potentially including clinical, research or development matters. Each JOT will develop and present to the JSC for approval specific plans for achieving its respective objectives and will be responsible for performing any duties assigned to such JOT by the JSC. Each JOT may form additional sub-groups as such JOT deems appropriate.

7.9.3 Votes; Disputes

Each JOT shall decide by vote on any subject matter within the decision-making authority of such JOT by a reasonable process to be established by the JOT. If a JOT is unable to reach a unanimous decision on any matter that is subject to the JOT's decision-making authority within [**] days after the JOT first fails to reach consensus regarding such matter (or

such later date as may be mutually acceptable to the Parties and the Foundation), then such matter shall be resolved by the JSC.

7.9.4 Joint IP Team

The JSC shall form a JIPT consisting of at least [**] from each Party's patent or legal department. It shall be the responsibility of the JIPT to (i) determine the need to license Third Party Patent Rights that are required or useful for the development and commercialization of Products and, subject to Section 11.5.4, the allocation of costs between the Parties in connection therewith, (ii) serve as a forum for the communication between the Parties regarding the Handling of Patent Rights, including but not limited to, with respect to any Joint Patent Right, determining which Party shall have the first right to Handle such Joint Patent Right, (iii) determine strategy for Handling Patent Rights, (iv) discuss the actual or suspected infringement that is the subject of an Infringement Notice, (v) determine the geographic scope of patent filings, including foreign filing decisions at the end of the priority year, PCT national/regional phase and European validations, and (vi) determine any matter regarding the enforcement or defense of the PTC Patent Rights, Roche Patent Rights or Joint Patent Rights. The JIPT shall meet as required to fulfill its duties as set forth in Sections 11.5.4, 15.5 and 15.8, or as otherwise mutually agreed by the Parties, and shall participate in and report to regular meetings of the JSC as requested by the JSC. The JIPT shall coordinate with Foundation to satisfy Foundation's right, pursuant to Section 6.2(a) of the SRA to comment and consultation on all filing, prosecution and maintenance activities with respect to any Patent Right that claims or covers any SRA Compound, and shall promptly provide Foundation with copies of all summaries of JIPT meetings that are prepared. If the JIPT is unable to resolve any dispute, or unanimously agree on any other matter before it, then, notwithstanding anything to the contrary in Section 7.9.3, such dispute or other matter shall be referred to the chief patent counsels of each Party or their designees to be resolved by negotiation in good faith as soon as practicable but in no event later than [**] days (or sooner if necessity so dictates) after referral. If the chief patent counsels or their designees are unable to resolve such matter, then Roche shall have final decision-making authority except as to (a) the allocation of costs under clause (i) of this Section 7.9.4, and (b) any Consensus Matter as set forth in Section 7.7.3. For clarity, the authority of the JIPT shall be subject to the rights of Foundation pursuant to Section 6.2(c) of the SRA.

7.10 Alliance Director

PTC, Roche and the Foundation shall each appoint an Alliance Director. The Alliance Directors shall be the point of contact within each Party and the Foundation with responsibility for facilitating communication and collaboration between PTC, Roche and the Foundation. They are permanent participants of the JSC meetings and may attend JOT meetings as appropriate. The Alliance Directors shall facilitate resolution of potential and pending issues and potential disputes to enable the JSC to reach consensus and avert escalation of such issues or potential disputes.

7.11 Limitations of Authority

The JSC shall have no authority to amend or waive any terms of this Agreement or the SRA.

7.12 Expenses

Each Party and the Foundation shall be responsible for its own expenses including travel and accommodation costs incurred in connection with the JSC and any JOT.

7.13 Lifetime

The JSC shall end upon the later to occur of (i) the conclusion of all research and development activities to be performed hereunder or (ii) the First Commercial Sale of the first Product in the second Major Country.

7.14 Appointment of JSC Members, JOT Members and Alliance Directors

The appointment of members of the JSC, any JOT and the Alliance Directors is a right of each Party and the Foundation and not an obligation and shall not be a "deliverable" as defined in EITF Issue No. 00-21. Each Party and the Foundation shall be free to determine not to appoint members to the JSC and any JOT and not to appoint an Alliance Director, and at any time during the Agreement Term and for any reason, either Party shall have the right to withdraw from participation in the JSC and any JOT and to remove its Alliance Director upon written notice to the other Party and the Foundation, or the Parties, as applicable, which notice shall be effective immediately upon receipt. If a Party or the Foundation ("**Appointing Party**") does not appoint members of the JSC or any JOT or an Alliance Director, or withdraws from the JSC or any JOT or removes its Alliance Director, it shall not be a breach of this Agreement, nor shall there be any associated penalty due nor shall there be any impact on the consideration otherwise provided for or due to the Appointing Party under this Agreement, and unless and until such persons are again appointed: (i) the other Party and the Foundation, or the Parties, as applicable, without regard to the provisions of this Article 7 with respect to voting, quorum or dispute resolution, may discharge the roles of the JSC, any JOT and the Alliance Director for which appointments were not made or with respect to which a withdrawal or removal has occurred by the Appointing Party (including without limitation, where the Appointing Party has not made appointments to the JSC or has withdrawn from the JSC, making all decisions within the decision-making authority of the JSC, which decisions shall be binding thereafter) and (ii) where the Appointing Party has not made appointments to the JSC or has withdrawn from the JSC, the Appointing Party shall not participate in any meetings of the JSC and shall not have the right to approve the minutes of any JSC meeting. If, at any time after the Effective Date, a Party or the Foundation has not appointed or has pursuant to this Section 7.14 withdrawn from the JSC or any JOT or removed its Alliance Director, and such Party or the Foundation wishes to resume participating in the JSC or any JOT or re-appoint its Alliance Director, such Party or the Foundation shall notify the other Party and the Foundation, or the Parties, as applicable, in writing and, thereafter, such Party's or the Foundation's designees shall be entitled to attend any subsequent meeting of the JSC or any JOT and to participate in the activities of, and decision-making by, the JSC or any JOT, and such Party's or Foundation's Alliance Director shall resume his or her duties, in each case as provided in this Article 7 as if a failure to appoint or submitting the withdrawal notice had not occurred.

7.15 Change of Control

If there is a Change of Control of PTC, PTC shall provide prompt written notice thereof to Roche; provided that PTC may (but shall not be obligated to) notify Roche on a confidential basis of a contemplated Change of Control prior to the effective date of such Change of Control (the "**Change of Control Notice**"). Within [**] days after receipt of a Change of Control Notice (the "**Election Period**"), Roche shall provide PTC with written notice of its election to proceed with one of the following mutually exclusive options: (i) to continue the Research Program pursuant to this Agreement and the Research Plan, (ii) to exclude PTC from participation in the JSC and any JOT and thereafter internalize at Roche all activities allocated to PTC under the Research Plan, or (iii) to terminate this Agreement in accordance with Section 20.3.2; provided that regardless of the option elected, such election shall become effective only upon or after the effective date of such Change of Control of PTC. If Roche fails to provide a written election under the preceding sentence within the Election Period, then Roche shall be deemed to have elected to proceed under Section 7.15(i). If Roche makes a written election pursuant to Section 7.15(ii) within the Election Period, then the Quarterly R&D Fees with respect to Research Program activities conducted by PTC after the effective date of the Change of Control shall be adjusted accordingly to reflect the change in activities. The Parties acknowledge and agree that if Roche makes a written election within the Election Period pursuant to Section 7.15(ii) or Section 7.15(iii), PTC shall have no obligation to transfer the AS Assay to Roche, and Roche will not obtain any right or license with respect to the AS Assay or any intellectual property right therein.

8. Supply

8.1 Research Supply of Product

All Product for use during the Research Program shall be supplied in accordance with the Research Plan and accompanying budget.

8.2 Clinical Supply of Product

8.2.1 Roche Responsibility

Roche shall be solely and exclusively responsible at its own expense for the manufacture and supply of clinical supplies of Product.

8.2.2 Technology Transfer

PTC shall initiate as soon as practicable and within [**] Business Days after the Effective Date a Know-How transfer (in accordance with the guidelines set forth in Appendix 8.2.2) to Roche to enable Roche to manufacture Product. If Roche provides PTC with reasonable notice requesting PTC to provide Roche or its Affiliates with technical assistance in transferring technology required for the manufacture of a Compound at a Roche Group manufacturing facility or subcontractor, then PTC shall provide [**] of up to [**] days duration of one FTE to provide such services [**]. If additional technical assistance is required, then PTC shall provide such assistance to Roche at the FTE Rate and Roche shall reimburse PTC for

reasonable out-of-pocket expenses, such travel and lodging, incurred in providing such assistance.

8.3 Commercial Supply of Product

Roche shall be solely and exclusively responsible at its own expense for the commercial manufacture and commercial supply of Product for sale in the Territory, either by itself or through Third Parties.

9. Regulatory

9.1 Responsibility

All regulatory responsibilities with respect to SRA Product are subject to the SRA. Roche, at its sole cost, shall pursue all regulatory affairs related to Product in the Territory, including without limitation the preparation and filing of applications for Regulatory Approval, as well as any or all governmental approvals required to develop, have developed, make, have made, use, have used, manufacture, have manufactured, import, have imported, sell and have sold Products. Roche shall be responsible for pursuing, compiling and submitting all regulatory filing documentation, and for interacting with Regulatory Authorities, for all Products in all countries in the Territory. Roche or its Affiliates shall own and file in their discretion all regulatory filings and Regulatory Approvals for all Products in all countries of the Territory. Roche shall supply PTC with a copy of all material communications related to Products to or from the Regulatory Authorities for all Major Countries.

Roche, at its sole cost, shall report to appropriate authorities in accordance with local requirements or Laws all adverse events related to use of Products in the Territory.

PTC shall transfer and assign to Roche any regulatory dossiers containing information necessary or useful to Roche in connection with its regulatory filings for all Products, including, but not limited to clinical trial dossiers, regulatory correspondence, Regulatory Authority meeting minutes and study reports from completed non-clinical studies. For all completed study reports, PTC shall provide necessary documentation to confirm data reliability, as required by Article 43 of the Japanese Pharmaceutical Affairs Law Enforcement Regulations and related notifications, including, but not limited to original author signatures, raw data lists, GLP and GCP compliance information. All documentation is to be provided in English.

9.2 Reporting Adverse Events

9.2.1 Report

Each Party agrees to inform the other about serious adverse events occurring or having occurred in connection with the use of Product that comes into its knowledge. The Parties agree to handle data and information about adverse events occurring or having occurred in connection with the use of Product according to the Guidelines in the respective territory, for example, those recited in the FDCA and the similar requirements of the Canadian or European Regulatory Authorities and/or requirements of any other relevant Regulatory Authority in the Territory.

9.2.2 Clinical Safety Database

Adverse events related to the use of Product in the Territory shall be logged in a single database, centralized, held and owned by Roche.

9.2.3 Pharmacovigilance Agreement

The Parties shall execute a separate pharmacovigilance agreement if required specifying the procedures and timelines for compliance with applicable Laws and regulations pertaining to safety reporting or Products and their related activities.

10. Commercialization

Subject to the SRA, Roche, at its own expense, shall have sole responsibility and decision-making authority for the marketing, promotion, sale and distribution of Products in the Territory. During the Agreement Term, for so long as any member of the Roche Group is conducting commercialization activities with respect to any Product hereunder, Roche shall provide PTC and Foundation with written reports, at least [**], summarizing the progress of such commercialization activities. In addition to such reports, at the request of PTC or Foundation from time to time following dissolution of the JSC, representatives of Roche shall meet, in person or via telephone, with representatives of PTC and Foundation at reasonable intervals for informal discussions regarding Roche's commercialization activities with respect to all Products then being commercialized by a member of the Roche Group; provided, however, that the foregoing shall not apply with respect to PTC if PTC or any PTC Affiliate is then marketing a product for the same indication as a Product then being commercialized by a member of the Roche Group.

11. Payment

11.1 Research Recognition Payment

Within [**] Business Days after the Effective Date and receipt of the invoice set forth in Appendix 11.1 (which shall be deemed issued to Roche on the Execution Date) from PTC, Roche shall make an unconditional, non-refundable, non-creditable payment to PTC of thirty million US dollars (US$ 30,000,000) in recognition of PTC's research efforts prior to the Effective Date. This Agreement, including without limitation Section 20.5 hereof, shall terminate in its entirety and shall be void and of no further force or effect if Roche fails to make the payment required by this Section 11.1 within such [**] Business Day period.

11.2 Research and Development Funding.

(a) Within [**] days after the end of each Calendar Quarter, PTC shall provide an invoice to Roche for the Quarterly R&D Fees actually incurred during such Calendar Quarter, and in prior periods with respect to amounts not yet invoiced or paid, and Roche shall pay PTC such Quarterly R&D Fees within [**] days after receipt thereof.

(b) Roche shall not be obligated to pay Quarterly R&D Fee amounts in excess of [**] percent ([**]%) above the amounts set forth in the then-current Research Plan as approved by the JSC, or in the then-current SRA Development Plan or New Product Development Plan, as applicable, unless such amounts have been preapproved by Roche in writing.

(c) If the Foundation performs any work or provides or procures any services as set forth in the Research Plan, SRA Development Plan or New Product Development Plan, then the Foundation shall provide Roche with an invoice setting forth all costs incurred by the Foundation in connection with such work or services, including out-of-pocket costs, internal costs and a reasonable allocation of overhead, and providing reasonable documentation thereof, and Roche shall pay Foundation such invoiced amount within [**] days after receipt thereof.

11.3 Research and Development Event Payments

Roche shall pay up to a total of [**] US dollars (US$ [**]) in relation to the achievements of events with respect to each Product. The payments under this Section 11.3 (each, an "**R&D Event Payment**") shall be paid by Roche according to the following schedule of research and development events (each, an "**R&D Event**"):

R&D Event		R&D Event Payment
(a)	[**]	[**][**]
(b)	[**]	[**]
(c)	[**]	[**]
(d)	[**]	[**]
(e)	[**]	[**]
(f)	[**]	[**]
(g)	[**]	[**]
(h)	[**]	[**]
(i)	[**]	[**]
(j)	[**]	[**]
	TOTAL	[**]

[**].

As used in the chart above, [**].

Each R&D Event Payment shall be non-refundable and non-creditable, and shall be paid no more than once, for the first applicable Product reaching the applicable R&D Event; provided, however, that if an R&D Event Payment is paid for a Product and such Product subsequently is withdrawn from development for any reason, then such R&D Event Payment shall be creditable against the analogous R&D Event Payment that would be due for a subsequent Product to reach such R&D Event; that is, if the subsequent Product replaces the previous Product with respect to the R&D Event Payments. For example, if (i) [**] in accordance with the above table.

If a given Product is developed and continues to be developed (and does not fail), and one or more additional Product(s) are also developed [**], then no payments with respect to all R&D Events achieved by each subsequent Product shall be made until such time [**]. For clarity, upon [**], all payments with respect to R&D Events achieved by such subsequent Product shall be due and payable.

The achievement of an R&D Event for a Product shall result in a simultaneous obligation to pay the R&D Event Payment for each antecedent R&D Event that has not been previously paid for such Product, unless such Product is a replacement of a Product that was withdrawn from development as set forth above. For example, on a Product-by-Product and a country-by-country or regional basis, the achievement of an R&D Event in any of rows [**]shall result in a simultaneous obligation to pay the relevant earlier R&D Event Payment in rows [**], as applicable, that had not previously been paid.

Upon achievement by or on behalf of any member of the Roche Group of any of the R&D Events, Roche shall promptly (but in no event more than [**] days after achievement thereof) notify PTC and shall pay to PTC all corresponding R&D Event Payments within [**] days after (x) occurrence of the applicable R&D Event and (y) receipt of an invoice from PTC.

11.4 Sales-Based Events

Roche shall pay to PTC up to a total of [**] US dollars (US$ [**]) based on aggregate Calendar Year Net Sales of a Product in the Territory:

Net Sales Threshold	Payment
Total Calendar Year Net Sales in the Territory of a Product exceed US$ [**]	US$[**]
Total Calendar Year Net Sales in the Territory of a Product exceed US$ [**]	US$[**]
Total Calendar Year Net Sales in the Territory of a Product exceed US$ [**]	US$[**]
Total Calendar Year Net Sales in the Territory of a Product exceed US$ [**]	US$[**]
TOTAL	**US$[**]**

Each of the sales-based event payments shall be paid no more than once during the Agreement Term, at first occurrence of the event for the Product in the Territory first reaching the respective Net Sales Threshold, irrespective of whether or not the previous sales-based event payment was triggered by the same or by a different Product, and shall be non-refundable and non-creditable. Upon achievement by the Roche Group of any of the foregoing sales events, Roche shall promptly (but in no event more than [**] days after achievement thereof) notify PTC, and shall pay PTC the corresponding sales event payment within [**] days after receipt of an invoice from PTC.

11.5 Royalty Payments

11.5.1 Royalty Term

Royalties shall be payable by Roche on Net Sales of Products on a Product-by Product basis until the expiry of the Royalty Term. Thereafter, the licenses granted to Roche and its Affiliates shall be fully paid-up, royalty-free and non-exclusive with respect to such Product in such country, on a Product-by-Product and country-by-country basis.

The following royalty rates shall apply to the respective tiers of aggregate Calendar Year Net Sales of a Product per area of the Territory, on an incremental basis, as follows:

Tier of Calendar Year Worldwide Net Sales in [**] US$	Percent (%) of Net Sales
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

For example, if Net Sales of a Product for a given Calendar Year are US$[**], then the royalties applicable on such Net Sales of such Product for that year shall be calculated as follows:

[**].

For the purpose of calculating royalties of a Product, Calendar Year Net Sales shall be subject to the adjustments set forth elsewhere in this Agreement.

11.5.2 Combination Product

If a Product is sold as part of a Combination Product in a country, the Net Sales of the Product, for the purposes of determining payments based on Net Sales, shall be determined by multiplying the Net Sales of the Combination Product in such country, during the applicable Net Sales reporting period, by the fraction, A/(A+B), where:

A is the average sale price of the Product by members of the Roche Group when sold separately in finished form in such country and B is the average sale price by members of the Roche Group of the other product(s) included in the Combination Product when sold separately in finished form in such country, in each case during the applicable Net Sales reporting period or, if sales of both the Product and the other product(s) did not occur in such period, then in the most recent Net Sales reporting period in which sales of both occurred.

If the Product is sold as part of a Combination Product and is sold separately in finished form in such country, but the other product(s) included in the Combination Product are not sold separately in finished form in such country, the Net Sales of the Product, for the

purposes of determining payments based on Net Sales, shall be determined by multiplying the Net Sales of the Combination Product in such country by the fraction C/D where:

C is the average sale price, in such country, of the Product contained in such Combination Product when sold separately and D is the average sale price, in such country, for the Combination Product, in each case during the applicable Net Sales reporting period.

If the Product is not sold separately in finished form in the country, but all of the other product(s) included in the Combination Product in such country are sold separately, the Net Sales of the Product, for the purposes of determining payments based on Net Sales, shall be determined by multiplying the Net Sales of the Combination Product in such country by the fraction (D-E)/D, where:

D is the average sale price, in such country, of the Combination Product, and E is the average sale price of the other product(s) included in the Combination Product in finished form in such country, in each case during the applicable Net Sales reporting period.

If Net Sales of the Product when included in a Combination Product cannot be determined using the methods above, Net Sales for the purposes of determining payments based on Net Sales shall be calculated by multiplying the Net Sales of the Combination Product by the fraction of F/(F+G) where:

F is the fair market value of the Product and G is the fair market value of all other pharmaceutical product(s) included in the Combination Product as reasonably determined in good faith by the Parties.

Where the preceding sentence is applicable, Roche shall in good faith propose to PTC an allocation of relative value of the Product and all other product(s) included in the Combination Product, PTC shall in good faith consider such proposal, and the Parties shall seek to reach agreement on such allocation. If the Parties are unable to reach such agreement within **[**]** days after Roche provides such proposal, the issue shall be referred for binding resolution to a mutually agreeable individual (not affiliated with either Party) with expertise in the marketing and sales of similar pharmaceutical products (including experience in pricing and reimbursement), such resolution to occur within **[**]** days after such referral.

11.5.3 No Valid Claim

Notwithstanding the foregoing, the royalty rate applicable to a Product sold in any country in the Territory shall be reduced to **[**]** percent (**[**]**%) of the rate otherwise payable during any portion of the Royalty Term when there is no Valid Claim of the PTC Patent Rights, Roche Product Patent Rights or Joint Patent Rights Covering such Product in such country; provided that such reduction shall not apply during any portion of the Royalty Term when such Product is entitled to Marketing Exclusivity in such country and there is no Generic Competition in such country.

11.5.4 Third Party Payments

The Parties, working through the JIPT, shall determine whether any Patent Rights Controlled by a Third Party are necessary or useful for the development and commercialization of Products. Roche and PTC shall each bear [**] percent ([**]%) of costs (including without limitation upfront payments, milestones and royalties) with respect to any license for any Third Party Patent Right that the JIPT (or Roche in its exercise of its final decision-making authority thereunder) reasonably determines is necessary for the development and commercialization of Products, and shall agree in good faith through the JIPT on the allocation of such costs with respect to any license for any Third Party Patent Right that is useful (but not necessary) for the development and commercialization of Products; provided that in no event shall PTC be required to pay any such costs except through a deduction from royalties payable by Roche to PTC hereunder, and no such royalty payable shall be reduced as a result of the allocation agreed by the JIPT in accordance with this Section 11.5.4 by more than [**] percent ([**]%) of the amount otherwise due to PTC with respect to the applicable Net Sales.

11.5.5 Anti-Stacking

In no event shall the combined effect of the applicable reductions pursuant to Sections 11.5.3 and 11.5.4 reduce the royalties payable to PTC below [**] percent ([**]%) of the amount otherwise payable to PTC pursuant to Section 11.5.1 or 11.5.2, as applicable. If the amount of the reduction Roche is otherwise entitled to pursuant to Sections 11.5.3 and 11.5.4 is limited by the preceding sentence, Roche shall be entitled to deduct amounts from any subsequent royalty payment(s) (subject to the [**] percent ([**]%) floor in the preceding sentence) until the entire amount of the reduction to which Roche is otherwise entitled has been so deducted.

11.5.6 Sales Prior to Regulatory Approval

If any member of the Roche Group sells Product(s) to any Third Party prior to Regulatory Approval for more than nominal consideration ("**Pre-Approval Sales**"), then royalties shall be payable in accordance with Sections 11.5.1 through 11.5.5 with respect to Net Sales received in connection with such Pre-Approval Sales of Product(s) in the Territory by any member of the Roche Group on a country-by-country basis commencing upon the first such Pre-Approval Sale of such Product in any country in the Territory and continuing until the First Commercial Sale of such Product in such country in the Territory.

11.6 Payments to Foundation

PTC shall be solely responsible for making all payments due to Foundation under the SRA.

11.7 Diagnostic Product

The provisions of Sections 11.3, 11.4 and 11.5 shall not apply to a diagnostic Product. If Roche desires to research, develop or commercialize a diagnostic Product in the Territory in the Field, then Roche shall have the right to acquire all necessary rights from PTC and the Parties

shall negotiate in good faith appropriate economic terms for Roche's development or commercialization of such diagnostic Product.

12. Accounting and reporting

12.1 Timing of Payments

Roche shall calculate royalties on Net Sales quarterly as of March 31, June 30, September 30 and December 31 (each being the last day of an "**Accounting Period**") and shall pay royalties on Net Sales within [**] days after the end of each Accounting Period in which such Net Sales occur.

12.2 Late Payment

Any payment under this Agreement that is not paid on or before the date such payment is due shall bear interest, to the extent permitted by applicable Law, at [**] above the average one-month Euro Interbank Offered Rate (EURIBOR), as reported by Reuters from time to time, calculated on the number of days such payment is overdue. In addition, Roche shall reimburse PTC for all costs and expenses, including without limitation attorneys' fees and legal expenses, incurred in the collection of late payments; provided that the foregoing shall not apply with respect to payments disputed in good faith by Roche unless PTC is successful in such dispute or Roche ceases to dispute such payments.

12.3 Method of Payment

Royalties on Net Sales and all other amounts payable by Roche hereunder shall be paid by Roche in US Dollars to account(s) designated by PTC.

12.4 Currency Conversion

When calculating Net Sales of any royalty bearing Product that occurs in currencies other than US$, Roche shall convert the amount of such sales in currencies other than Swiss Francs into Swiss Francs and then into US$ using for internal foreign currency translation Roche's then current standard practices actually used on a consistent basis in preparing its audited financial statements.

Upon converting the amount of sales into Swiss Francs, Roche shall convert into US$ (or other currency), using the daily rate (currently Reuters) at the last working day for the applicable period.

12.5 Reporting

With each payment Roche shall provide PTC in writing for the relevant Calendar Quarter on a Product-by-Product basis the following information:

a) Sales in Swiss Francs on a country-by-country basis;

b) Deductions from Sales to calculate Net Sales;

c) Net Sales in Swiss Francs on a country-by-country basis;

d) Adjustments made pursuant to Sections 11.5.2 - 11.5.4 in Swiss Francs;

e) Net Sales after adjustments made pursuant to Sections 11.5.2 - 11.5.4, in Swiss Francs;

f) Total Net Sales in the Territory in Swiss Francs;

g) Total Net Sales in the Territory in US$;

h) Total royalty payable to PTC in US$; and

i) Exchange rates used for the conversions of Nets Sales from Swiss Francs to US$ made under Section 12.4 above.

13. Taxes

The royalties, milestones, Quarterly R&D Fees and other amounts payable by Roche to PTC pursuant to this Agreement ("**Payments**") shall not be reduced on account of any taxes unless required by applicable Law. PTC alone shall be responsible for paying any and all taxes (other than withholding taxes required by applicable Law to be paid by Roche) levied on account of, or measured in whole or in part by reference to, any Payments it receives. Roche shall deduct or withhold from the Payments any taxes that it is required by applicable Law to deduct or withhold. Notwithstanding the foregoing, if PTC is entitled under any applicable tax treaty to a reduction of rate of, or the elimination of, applicable withholding tax, it may deliver to Roche or the appropriate governmental authority (with the assistance of Roche to the extent that this is reasonably required and is expressly requested in writing) the prescribed forms necessary to reduce the applicable rate of withholding or to relieve Roche of its obligation to withhold tax, and Roche shall apply the reduced rate of withholding, or dispense with withholding, as the case may be; provided that Roche has received evidence, in a form satisfactory to Roche, of PTC's delivery of all applicable forms (and, if necessary, its receipt of appropriate governmental authorization) at least [**] Business Days prior to the time that the Payments are due. If, in accordance with the foregoing, Roche withholds any amount, it shall pay to PTC the balance when due, make timely payment to the proper taxing authority of the withheld amount, and send to PTC proof of such payment within [**] days following that payment.

14. Auditing

14.1 PTC Right to Audit

Roche shall keep, and shall require its and its Affiliates and Sublicensees to keep, full, true and accurate books of account containing all particulars that may be necessary for the purpose of calculating all royalties payable under this Agreement. Such books of accounts shall be kept at their principal place of business. At the expense of PTC, PTC has the right to engage an independent, certified public accountant reasonably acceptable to both Parties to perform, on

behalf of PTC an audit of such books and records of members of the Roche Group that are deemed necessary by such independent public accountant to report on Net Sales of Product for

the period or periods requested by PTC and the correctness of any report or payments made under this Agreement.

Upon timely request and at least [**] working days' prior written notice from PTC, such audit shall be conducted in the countries specifically requested by PTC, during regular business hours in such a manner as to not unnecessarily interfere with Roche's normal business activities, and shall be limited to results in the [**] Calendar Years prior to audit notification.

Such audit shall not be performed more frequently than [**] nor more frequently than [**] with respect to records covering any specific period of time.

All information, data and documents herein referred to shall be used only for the purpose of verifying royalty statements and all other amounts due hereunder, shall be treated as Roche Confidential Information subject to the obligations of this Agreement and need neither be retained more than the longer of [**] after completion of an audit hereunder, if an audit has been requested; nor more than [**] years from the end of the Calendar Year to which each shall pertain.

The auditors shall not interpret the Agreement. Draft audit reports shall be provided to both PTC and Roche by the auditor for review and comment by PTC and Roche and the final audit report shall be shared by PTC and Roche.

14.2 Over- or Underpayment

If an audit reveals an underpayment by Roche, then Roche shall pay PTC for the amount of the underpayment within [**] days after receipt of the final audit report, with interest thereon as set forth in Section 12.2. If an audit reveals an overpayment by Roche, Roche shall have the right to credit the amount of such overpayment against the next royalty payment payable to PTC hereunder. PTC shall pay for the audit costs, except that if an audit reveals an underpayment in excess of [**] percent ([**]%) of the aggregate amount of royalty payments owed with regard to the royalty statements subject to the audit, then Roche shall reimburse PTC for such audit costs concurrently with its payment of the amount of the underpayment.

14.3 Duration of Audit Rights

The failure of PTC to request verification of any royalty calculation within the period during which corresponding records must be maintained under this Article 14 will be deemed to be acceptance of the royalty payments and reports.

15. Intellectual Property

15.1 Ownership of Inventions

PTC shall own all PTC Inventions, Roche shall own all Roche Inventions, and PTC and Roche shall jointly own all Joint Inventions. The determination of inventorship for Inventions shall be in accordance with US inventorship Laws. PTC and Roche each shall require all of its employees and contractors to assign all inventions related to Products made by them to Roche and PTC, as the case may be. In the case of all others acting in the performance of the Research

Program on behalf of such Party, such as agents or non-employees working for non-profit academic institutions, such others shall also be obligated under a written agreement or other binding obligation that meets the criteria of the preceding sentence or includes an exclusive license grant to such Party, or as such Party shall direct, unless otherwise approved by the JSC; provided, that in the case of government funded or nonprofit entities, such Party's obligation shall be limited to using Commercially Reasonable Efforts to negotiate for at least an exclusive option to license such Invention, subject to any requirement of Law (including without limitation mandatory licenses and other rights granted to government entities).

15.2 German Statute on Employee's Inventions

In accordance with the German Statute on Employees' Inventions, each Party agrees to claim the unlimited use of any Invention conceived, reduced to practice, developed, made or created in the performance of, or as a result of, the Research Program or the Product Development Program by its German employees or any other German person acting on its behalf. The Party which is the ultimate assignee of the German employee's or other person's Invention under this Agreement shall pay any royalty or other compensation payable to the employee or other person, and the Parties agree that the Party which is the ultimate assignee must agree to the royalty or other compensation negotiated with the employee or other person.

15.3 Prosecution of Patent Rights Claiming PTC Inventions

Subject to Section 7.9.4, PTC shall (i) Handle all PTC Patent Rights, (ii) consult with Roche as to the Handling of such PTC Patent Rights, and (iii) furnish to Roche copies of all documents relevant to any such Handling. PTC shall furnish such documents and consult with Roche in sufficient time before any action by PTC is due to allow Roche to provide comments thereon, which comments PTC must consider. At PTC's reasonable request, Roche shall cooperate in all reasonable ways with the Handling of all PTC Patent Rights. PTC shall not discontinue prosecution or maintenance of any PTC Patent Right (including but not limited to selection of countries for foreign filing or entry into the PCT National Stage) without at least [**] days' prior written notice to Roche. If PTC decides to discontinue Handling any PTC Patent Right, Roche shall have the option to assume responsibility for Handling such PTC Patent Right and, in such case, except for a change in responsibility for Handling such PTC Patent Right under this Section 15.3, no changes in ownership or licensing terms pertaining to any PTC Patent Right shall occur. For clarity, the rights of the Parties pursuant to this Section 15.3 shall be subject to the rights of Foundation pursuant to Section 6.2(c) of the SRA.

15.4 Prosecution of Patent Rights Claiming Roche Inventions

Subject to Section 7.9.4, Roche shall (i) Handle all Roche Patent Rights, (ii) consult with PTC as to the Handling of such Roche Patent Rights, and (iii) furnish to PTC copies of all documents relevant to any such Handling. Roche shall furnish such documents and consult with PTC in sufficient time before any action by Roche is due to allow PTC to provide comments thereon, which comments Roche must consider. At Roche's expense and reasonable request, PTC

shall cooperate, in all reasonable ways with the Handling of all Roche Patent Rights. Roche shall not discontinue Handling any Roche Patent Right (including but not limited to selection of countries for foreign filing or entry into the PCT National Stage) without at least [**] days' prior

written notice to PTC. If Roche decides to discontinue Handling any Roche Patent Right, PTC shall have the option to assume responsibility for prosecuting and maintaining such Roche Patent Right, at PTC's sole expense, and in such case, except for a change in responsibility for Handling such Roche Patent Right under this Section 15.4, no changes in ownership or licensing terms pertaining to any Roche Patent Right shall occur. For clarity, the rights of the Parties pursuant to this Section 15.4 shall be subject to the rights of Foundation pursuant to Section 6.2(c) of the SRA.

15.5 Prosecution of Patent Rights Claiming Joint Inventions

On a Joint Invention-by-Joint Invention basis, the JIPT shall determine which Party shall be responsible for Handling each Joint Patent Right with respect to such Joint Invention. Each Party shall keep the other Party informed of the status of all pending applications disclosing the Joint Patent Rights for which it has Handling responsibility, and shall consider in good faith all of the other Party's comments regarding any aspect of such patent Handling. Neither Party shall discontinue Handling of any Joint Patent Right (including but not limited to selection of countries for foreign filing or entry into the PCT National Stage) without at least [**] days' prior written notice to the other Party. If a Party decides to discontinue Handling any Joint Patent Right, then the other Party shall have the option to continue to Handle such Joint Patent Right, at such other Party's sole expense, and in such case, except for the change in responsibility for Handling such Joint Patent Right under this Section 15.5, no changes in ownership or licensing terms pertaining to any Joint Patent Right shall occur.

15.6 Prosecution Costs

Except as otherwise expressly provided hereunder, Roche shall bear all out-of-pocket costs and expenses of Handling PTC Patent Rights, Roche Patent Rights and Joint Patent Rights, and shall reimburse PTC for any such costs PTC incurs in connection therewith within [**] days after receipt of an invoice from PTC therefor.

15.7 CREATE Act

It is the intention of the Parties that this Agreement is a "joint research agreement" as that phrase is defined in 35 USC §103(c)(3).

15.8 Infringement

Each Party shall promptly provide written notice ("**Infringement Notice**") to the other Party during the term of this Agreement of any (i) known infringement or suspected infringement by a Third Party of any PTC Patent Right, Roche Patent Right or Joint Patent Right, or (ii) known or suspected unauthorized use or misappropriation by a Third Party of any PTC Know-How, Roche Know-How or Joint Know-How, and shall provide the other Party with all evidence in its possession supporting such infringement or unauthorized use or misappropriation.

As soon as possible after receiving an Infringement Notice, the Parties will convene a meeting of the JIPT at which the JIPT may discuss in good faith all available evidence of such infringement, use or misappropriation, and the appropriate manner of addressing such infringement, use or misappropriation, including without limitation preventing or stopping

infringing activities (for example, by seeking a preliminary injunction), preserving the Parties' rights to past and future damages (for example, by sending a cease and desist letter) defending against declaratory judgment actions with respect thereto, or taking any other action, or no action, as the Parties shall determine. The JIPT shall take into account each Party's interest in formulating the response, if any, to infringement or threatened infringement of such PTC Patent Rights, Roche Patent Rights, or Joint Patent Rights or known or suspected unauthorized use or misappropriation of such PTC Know-How, Roche Know-How or Joint Know-How, including without limitation the relative merits of patent litigation versus the nature, scope and potential economic consequences of the infringement. Unless otherwise determined by the JIPT, Roche shall have the right initiate a suit or action to address such infringement, use or misappropriation, and shall and shall provide written notice to PTC of its decision ("**Suit Notice**") within [**] days after Roche provides or receives the Infringement Notice ("**Decision Period**").

If Roche decides to bring a suit or take action, once Roche provides Suit Notice, Roche may immediately commence such suit or take such action. In the event that Roche (i) does not in writing advise PTC within the Decision Period that Roche will commence suit or take action, or (ii) fails to commence suit or take action within a reasonable time after providing Suit Notice, PTC shall thereafter have the right to commence suit or take action in the Territory and shall provide written notice to Roche of any such suit commenced or action taken by PTC.

Upon written request, the Party bringing suit or taking action ("**Initiating Party**") shall keep the other Party informed of the status of any such suit or action and shall provide the other Party with copies, to the extent the Initiating Party is lawfully permitted to do so, of all substantive documents or communications filed in such suit or action. The Initiating Party shall have the sole and exclusive right to select counsel for any such suit or action.

The Initiating Party shall, except as provided below, pay all expenses of the suit or action, including without limitation the Initiating Party's attorneys' fees and court costs. Any damages, settlement fees or other consideration received as a result of such suit or action shall be allocated as follows:

(a) First, to reimburse the Initiating Party for its costs and, if any remains, to the other Party for any advisory counsel fees and costs; and

(b) Second, the balance, if any, shall be allocated [**] percent ([**]%) to the Initiating Party, and [**] percent ([**]%) to the other Party.

If the Initiating Party believes it is reasonably necessary or desirable to obtain an effective remedy, upon written request the other Party agrees to be joined as a party to the suit or action but shall be under no obligation to participate except to the extent that such participation is required as the result of its being a named party to the suit or action. At the Initiating Party's written request, the other Party shall offer reasonable assistance to the Initiating Party in connection therewith at no charge to the Initiating Party except for reimbursement of reasonable out-of-pocket expenses incurred by the other Party in rendering such assistance. The other Party shall have the right to participate and be represented in any such suit or action by its own counsel at its own expense.

The Initiating Party may settle, consent to judgment or otherwise voluntarily dispose of the suit or action ("**Settlement**") without the written consent of the other Party but only if such Settlement can be achieved without adversely affecting the other Party (including without limitation any of its Patent Rights or Know-How). If a Settlement could adversely affect the other Party, then the written consent of the other Party would be required, which consent shall not be unreasonably withheld.

15.9 Defense

If an action for infringement is commenced against either Party, its licensees or its sub-licensees related to PTC's conduct of the Research Program within the scope of the Research Plan or the discovery, development, manufacture, use or sale of a Product, then Roche shall defend such action at its own expense, and PTC shall assist and cooperate with Roche, at Roche's expense, to the extent necessary in the defense of such suit. Roche shall have the right to settle the suit or consent to an adverse judgment thereto, in its sole discretion, so long as such settlement or adverse judgment does not adversely affect the rights of PTC and its Affiliates (including without limitation any Patent Right or Know-How Controlled by any of them). Roche shall assume full responsibility for the payment of any award for damages, or any amount due pursuant to any settlement entered into by it with such Third Party.

If the manufacture, use, importation, offer for sale or sale of any Product pursuant to this Agreement results in any claim, suit or proceeding alleging patent infringement or trade secret misappropriation against PTC or a member of the Roche Group, then PTC or Roche, respectively, shall promptly notify the other Party hereto. The Parties shall cooperate with each other in connection with any such claim, suit or proceeding and shall keep each other reasonably informed of all material developments in connection with any such claim, suit or proceeding.

If a Third Party asserts that Patent Rights owned by or licensed to it are infringed by the development, manufacture, use, importation, offer for sale or sale of Products by a member of the Roche Group, or that its trade secrets were misappropriated in connection with such activity, then Roche shall have the exclusive right and responsibility to resolve any such claim, whether by obtaining a license from such Third Party, by defending against such Third Party's claims or otherwise, and shall be solely responsible for the defense of any such action, any and all costs incurred in connection with such action (including, without limitation, attorneys' and expert fees) and all liabilities incurred in connection therewith. Notwithstanding the above, Roche shall not enter into any settlement of any such claim without the prior written consent of PTC if such settlement would require PTC to be subject to an injunction or to make any monetary payment to Roche or any Third Party, or admit any wrongful conduct by PTC or its Affiliates, or would limit or restrict the claims of or admit any invalidity and/or unenforceability of any of the Patent Rights Controlled by PTC, or have any impact on activities outside the Field.

15.10 Common Interest Disclosures

With regard to any information or opinions disclosed pursuant to this Agreement by one Party to each other regarding intellectual property and/or technology owned by Third Parties, the Parties agree that they have a common legal interest in determining whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Research Program and/or

the development and/or commercialization of Compounds and/or Products, and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating to the conduct of the Research Program and/or the development and/or commercialization of Compounds and/or Products. The Parties anticipate that each of them will have legal counsel involved in such determinations. Accordingly, the Parties agree that all such information and materials obtained by PTC and Roche from each other will be used solely for purposes of the Parties' common legal interests with respect to the conduct of the Agreement. All information and materials will be treated as protected by the attorney-client privilege, the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party intends to waive or limit any privilege or immunity that may apply to the shared information and materials. Neither Party shall have the authority to waive any privilege or immunity on behalf of the other Party without such other Party's prior written consent, nor shall the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against any other Party.

15.11 Hatch-Waxman

Notwithstanding anything herein to the contrary, should a Party receive a certification for a Product pursuant to the Drug Price Competition and Patent Term Restoration Act of 1984 (Public Law 98-417, known as the Hatch-Waxman Act), as amended, or its equivalent in a country other than the US, then such Party shall immediately provide the other Party with a copy of such certification. Roche shall have [**] days after the date on which it receives or provides a copy of such certification to provide written notice to PTC ("**H-W Suit Notice**") whether Roche will bring suit, at its expense, within a [**] day period after the date of such certification. Should such [**] day period expire without Roche bringing suit or providing such H-W Suit Notice, then PTC shall be free to immediately bring suit in its name. The provisions of Section 15.8 (except for paragraphs 1-3 thereof) shall apply with respect to any such suit.

15.12 Patent Term Extensions

The Parties shall use Commercially Reasonable Efforts to obtain all available patent term extensions, adjustments or restorations, or supplementary protection certificates ("**SPCs**", and together with patent term extensions, adjustments and restorations, "**Patent Term Extensions**"). PTC shall execute such authorizations and other documents and take such other actions as may be reasonably requested by Roche to obtain such Patent Term Extensions, including designating Roche as its agent for such purpose as provided in 35 U.S.C. §156. All filings for such Patent Term Extensions shall be made by Roche; provided, that in the event that Roche elects not to file for a Patent Term Extension, Roche shall (a) promptly inform PTC of its intention not to file and (b) grant PTC the right to file for such Patent Term Extension. Each Party shall execute such authorizations and other documents and take such other actions as may be reasonably requested by the other Party to obtain such extensions. The Parties shall cooperate with each other in gaining patent term restorations, extensions and/or SPCs wherever applicable to PTC Patent Rights, Roche Patent Rights and Joint Patent Rights.

16. Representations and Warranties

16.1 Mutual Representations and Warranties

Each Party represents and warrants to the other Party as follows:

16.1.1 Authorization

The execution, delivery and performance of this Agreement by such Party and all instruments and documents to be delivered by such Party hereunder: (i) are within the corporate power of such Party; (ii) have been duly authorized by all necessary or proper corporate action; (iii) are not in contravention of any provision of the certificate of formation or limited liability company agreement of such Party; (iv) to the knowledge of such Party, will not violate any Law or regulation or any order or decree of any court of governmental instrumentality; (v) will not violate the terms of any indenture, mortgage, deed of trust, lease, agreement, or other instrument to which such Party is a party or by which such Party or any of its property is bound, which violation would have an adverse effect on the financial condition of such Party or on the ability of such Party to perform its obligations hereunder; and (vi) do not require any filing or registration with, or the consent or approval of, any governmental body, agency, authority or any other person, which has not been made or obtained previously (other than approvals required under the HSR Act, Regulatory Approvals required for the sale of Products and filings with Regulatory Authorities required in connection with Products).

16.1.2 Third Party Patent Rights

Such Party has no knowledge as of the Effective Date of the existence of any Valid Claim of any issued patent owned by or licensed to any Third Party that could prevent Roche from making, having made, using, offering for sale, selling or importing Product in the Territory.

16.1.3 Inventors

All of such Party's employees, officers and contractors have executed agreements requiring assignment to such Party of all Inventions made by such individuals during the course of and as a result of their association with such Party.

16.1.4 Grants

Such Party has the lawful right to grant the other Party and its Affiliates the rights and licenses described in this Agreement.

16.1.5 No Claims

As of the Effective Date, there are no claims or investigations (other than with respect to the Parties' HSR Act filings), pending or threatened against such Party or any of its Affiliates, at Law or in equity, or before or by any governmental authority, relating to the matters contemplated under this Agreement or that would materially adversely affect such Party's ability to perform its obligations hereunder.

16.1.6 No Conflict

Except for obligations under the SRA, neither such Party nor any of its Affiliates is or will be under any obligation to any person, contractual or otherwise, that is conflicting with the terms of this Agreement or that would impede the fulfillment of such Party's obligations hereunder.

16.1.7 No Debarment

Such Party has never been debarred under 21 U.S.C. §335a, disqualified under 21 C.F.R. §312.70 or §812.119, sanctioned by a Federal Health Care Program (as defined in 42 U.S.C. §1320 a-7b(f)), including without limitation the federal Medicare or a state Medicaid program, or debarred, suspended, excluded or otherwise declared ineligible from any other similar Federal or state agency or program.

16.2 Additional PTC Representations and Warranties

16.2.1 Safety Data

PTC has disclosed to Roche (i) the results of all preclinical testing of Products in its Control and (ii) all information in its Control concerning side effects, injury, toxicity or sensitivity reaction and incidents or severity thereof with respect to Product.

16.2.2 Ownership of Patent Rights

PTC is the exclusive owner of all right, title and interest in, or is the exclusive licensee of, the PTC Base Patent Rights. Appendix 1.62 contains a complete and accurate list of all patents and patent applications included in the PTC Base Patent Rights.

16.2.3 Validity of Patent Rights

As of the Effective Date, PTC is not in possession of information that could render invalid and/or unenforceable any claims that are in any of the PTC Base Patent Rights. PTC has no knowledge of any inventorship disputes concerning any PTC Base Patent Rights.

16.2.4 Ownership and Validity of Know-How

The PTC Know-How is legitimately in the possession of PTC and has not been misappropriated from any Third Party. PTC has taken reasonable measures to protect the confidentiality of its Know-How.

16.2.5 SRA

As of the Effective Date, the SRA is in full force and effect and neither PTC nor Foundation has been notified of any breach of the SRA or controversy concerning the performance of PTC and/or Foundation under the SRA.

16.3 Additional Roche Representation

Roche represents that it is the view of its professional advisors as of the Effective Date that no filing will be required under the HSR Act in connection with this Agreement.

17. Indemnification

17.1 Indemnification by Roche

(a) Roche shall indemnify, hold harmless and defend PTC and its directors, officers, employees and agents from and against any and all losses, expenses, cost of defense (including without limitation attorneys' fees, witness fees, damages, judgments, fines and amounts paid in settlement) and any amounts PTC becomes legally obligated to pay because of any claim or claims against it to the extent that such claim or claims arise out of Research Program or Product Development Program activities, or any other activities related to the Product (e.g., product liability claims), conducted by or on behalf of any member of the Roche Group, except to the extent such losses, expenses, costs and amounts are due to the gross negligence or willful misconduct or failure to act of PTC.

(b) Roche shall indemnify, hold harmless and defend the Foundation and its directors, officers, employees and agents from and against any and all losses, expenses, cost of defense (including without limitation attorneys' fees, witness fees, damages, judgments, fines and amounts paid in settlement) and any amounts the Foundation becomes legally obligated to pay because of any claim or claims against it to the extent that such claim or claims arise out of Research Program or Product Development Program activities, or any other activities related to the Product (e.g., product liability claims), conducted by or on behalf of any member of the Roche Group, except to the extent such losses, expenses, costs and amounts are due to the gross negligence or willful misconduct or failure to act of the Foundation. For clarity, nothing in this Section 17.1(b) or Sections 17.2 or 17.3 shall be interpreted as limiting PTC's obligations pursuant to the SRA, including its obligations pursuant to Section 8.1 of the SRA.

17.2 Indemnification by PTC

PTC shall indemnify, hold harmless and defend Roche and its directors, officers, employees and agents from and against any and all losses, expenses, cost of defense (including without limitation attorneys' fees, witness fees, damages, judgments, fines and amounts paid in settlement) and any amounts Roche becomes legally obligated to pay because of any claim or claims against it to the extent that such claim or claims arise out of activities related to the Research Program or Product Development Program activities or any other activities conducted by or on behalf of PTC relating to PTC's obligations under this Agreement, except to the extent such losses, expenses, costs and amounts are due to the gross negligence or willful misconduct or failure to act of any member of the Roche Group.

17.3 Procedure

In the event of a claim by a Third Party against any Person entitled to indemnification under this Agreement (in such capacity, the "**Indemnified Party**"), the Indemnified Party shall promptly notify the other Party (or the Foundation) (in such capacity, the "**Indemnifying Party**") in writing of the claim (it being understood that the failure by the Indemnified Party to give prompt notice of a Third Party claim as provided in this Section 17.3 shall not relieve the Indemnifying Party of its indemnification obligation under this Agreement except and only to the extent that such Indemnifying Party is actually prejudiced as a result of such failure to give prompt notice). Within [**] days after delivery of such notification, the Indemnifying Party may, upon written notice thereof to the Indemnified Party, undertake and solely manage and control, at its sole expense and with counsel reasonably satisfactory to the Indemnified Party, the defense of the claim. If the Indemnifying Party does not undertake such defense, the Indemnified Party shall control such defense. The Party (or the Foundation, as applicable) not controlling such defense shall cooperate with the other Party (or the Foundation, as applicable) and may, at its option and expense, participate in such defense; provided that if the Indemnifying Party assumes control of such defense and the Indemnified Party in good faith concludes, based on advice from counsel, that the Indemnifying Party and the Indemnified Party have conflicting interests with respect to such action, suit, proceeding or claim, the Indemnifying Party shall be responsible for the reasonable fees and expenses of counsel to the Indemnified Party solely in connection therewith. The Party (or the Foundation, if applicable) controlling such defense shall keep the other Party (or the Foundation, as applicable) advised of the status of such action, suit, proceeding or claim and the defense thereof and shall consider recommendations made by the other Party (or the Foundation, as applicable) with respect thereto. The Indemnifying Party shall not be liable for any litigation costs or expenses incurred by the Indemnified Party without the Indemnifying Party's written consent. The Indemnified Party shall not settle any such action, suit, proceeding or claim without the prior written consent of the Indemnifying Party, which shall not be unreasonably withheld, delayed or conditioned. Without the prior written consent of the Indemnified Party, the Indemnifying Party shall not settle any such action, suit, proceeding or claim, or consent to any judgment in respect thereof, that does not include a complete and unconditional release of the Indemnified Party from all liability with respect thereto, that imposes any liability or obligation on the Indemnified Party or that acknowledges fault by the Indemnified Party.

18. Disclaimer

EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS AGREEMENT, PTC AND ROCHE EACH DISCLAIM ALL WARRANTIES, WHETHER EXPRESS OR IMPLIED, WITH RESPECT TO EACH OF THEIR RESEARCH, DEVELOPMENT AND COMMERCIALIZATION EFFORTS HEREUNDER, INCLUDING, WITHOUT LIMITATION, WHETHER THE PRODUCTS CAN BE SUCCESSFULLY DEVELOPED OR MARKETED, THE ACCURACY, PERFORMANCE, UTILITY, RELIABILITY, TECHNOLOGICAL OR COMMERCIAL VALUE, COMPREHENSIVENESS, MERCHANTABILITY, NONINFRINGEMENT, OR FITNESS FOR ANY PARTICULAR PURPOSE WHATSOEVER OF THE PRODUCTS. IN NO EVENT SHALL THE PARTIES OR THE FOUNDATION BE LIABLE FOR SPECIAL, INDIRECT, INCIDENTAL OR CONSEQUENTIAL DAMAGES ARISING OUT OF THIS AGREEMENT BASED ON

CONTRACT, TORT OR ANY OTHER LEGAL THEORY. NOTHING IN THIS ARTICLE 18 IS INTENDED TO LIMIT OR RESTRICT (I) THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY OR FOUNDATION UNDER SECTIONS 15.9, 17.1 AND 17.2, OR (II) REMEDIES AVAILABLE TO EITHER PARTY OR THE FOUNDATION WITH RESPECT TO A BREACH OF SECTION 19.1.

19. Obligation Not to Disclose Confidential Information

19.1 Non-Use and Non-Disclosure

During the Term of this Agreement and for [**] years thereafter, a Receiving Party shall (i) treat Confidential Information provided by a Disclosing Party as it would treat its own information of a similar nature, (ii) take all reasonable precautions not to disclose such Confidential Information to Third Parties or Sublicensees without the Disclosing Party's prior written consent, and (iii) not use such Confidential Information other than for fulfilling its obligations or exercising its rights under this Agreement or the SRA.

19.2 Permitted Disclosure

Notwithstanding the obligation of non-use and non-disclosure set forth in Section 19.1, the Parties and the Foundation recognize the need for certain exceptions to this obligation, specifically set forth below, with respect to press releases, Patent Rights, publications, and certain commercial considerations. In addition, a Receiving Party shall not be bound by the obligation of non-disclosure set forth in Section 19.1 to the extent it is required to disclose Confidential Information of a Disclosing Party to comply with applicable Law, provided that the Receiving Party furnishes prompt notice (in no event less than [**] days unless otherwise required by applicable Law) to the Disclosing Party to enable it to resist or minimize the scope of such disclosure.

19.3 Disclosure to Foundation

To the extent reasonably necessary or useful to fulfill the requirements or accomplish the objectives of the SRA or this Agreement, each Party may disclose the other Party's Confidential Information to the Foundation and the Foundation's Confidential Information to the other Party, and the Foundation may disclose a Party's Confidential Information to the other Party.

19.4 Press Releases and Other Disclosures

The Parties and the Foundation will cooperate in the release of a joint press release, substantially in the form set forth in Appendix 19.4, as soon as practicable after the Effective Date. The Parties and the Foundation also recognize that each Party or the Foundation may from time to time desire to issue additional press releases and make other public statements or disclosures regarding the subject matter of this Agreement or the SRA (if the Disclosing Party is PTC or the Foundation). In such event, the Party desiring to issue an additional press release or make a public statement or disclosure (or the Foundation, as applicable) shall provide the other Party and the Foundation, or the Parties, as applicable, with a copy of the proposed press release, statement or disclosure for review, comment and approval at least [**] Business Days in advance (or such shorter period as would permit the publicizing Party (or the Foundation, as applicable) to comply with applicable Law), which advance approval shall not be unreasonably withheld, conditioned or delayed (except that neither Party nor the Foundation shall have any obligation to disclose Confidential Information except to the extent required or permitted pursuant to the other provisions of this Article 19). Each reviewing Party (and the Foundation, as applicable) shall notify the publicizing Party (or the Foundation, as applicable) within such [**] Business Days period (or such shorter period) of its comments and whether it approves such disclosure. It is agreed that each such disclosure shall only be done with such approval of each reviewing Party (and the Foundation, as applicable)

and no other public statement or disclosure concerning the existence or terms of this Agreement or the SRA shall be made, either directly or indirectly, by either Party or the Foundation, without first obtaining the written approval of the other Party and the Foundation, or the Parties, as applicable. Notwithstanding the foregoing provisions of this Section 19.4 or the other provisions of this Article 19, (i) a Party or the Foundation may make any disclosure or public announcement if the contents of such disclosure or public announcement have previously been made public other than through a breach of this Agreement by the issuing Party (or the Foundation, as applicable); (ii) if a Party or the Foundation reasonably determines that a public disclosure shall be required by Law, including without limitation in a public filing with the US Securities and Exchange Commission, such Party (or the Foundation, as applicable) may disclose the existence and terms of this Agreement and any material developments that occur under this Agreement where so required; provided that such Party (or the Foundation, as applicable) shall, to the extent practicable and permitted by applicable Law, notify the other Party and the Foundation, or the Parties, as applicable, and allow the other Party and the Foundation, or the Parties, as applicable to comment on the proposed disclosure, which comments shall be considered by the disclosing Party (or the Foundation, as applicable) in good faith; (iii) a Party or the Foundation may disclose the terms of this Agreement under obligations of confidentiality to *bona fide* potential or actual advisors, consultants, investors, acquirers, lenders, investment bankers or other potential financial partners in connection with such Party's (or the Foundation's) proposed financing or business combination activities, including without limitation any Reverse Merger; and (iv) Roche may disclose the terms of this Agreement to *bona fide* potential or actual Sublicensees, as reasonably necessary in connection with a permitted sublicense under the licenses granted in this Agreement.

19.5 Publications

During the Agreement Term, the following restrictions shall apply with respect to disclosure by any Party or the Foundation of the Confidential Information of the other Party, the Parties, or the Foundation, as applicable, relating to Compounds or Products in any publication or presentation.

a) Both Parties and the Foundation acknowledge that it is their policy for studies and results thereof to be registered and published in accordance with their internal guidelines. Roche, in accordance with its internal policies and procedures, shall have the right to publish all studies, Clinical Studies and results thereof on the clinical trial registries that are maintained by or on behalf of Roche. PTC shall not publish any studies, Clinical Studies or results thereof on its clinical trial registry; provided however, that Roche's clinical trial registry can be accessed via a link from PTC's clinical trial registry. The Foundation shall have the right to

publish results of the SRA Research as set forth in Section 5.4 of the SRA, subject to the procedures set forth in this Section 19.5, provided that such procedures do not prevent the Foundation from publishing or otherwise making available such results as contemplated by Section 5.4 of the SRA.

b) A Party or the Foundation (the "**Publishing Party**") shall provide the other Party and the Foundation, or the Parties, as applicable, with a copy of any proposed publication or presentation at least [**] days (or at least [**] days in the case of abstracts or oral presentations) prior to submission for publication by the Publishing Party or its Affiliates so as to provide the other Party and the Foundation, or the Parties, as applicable, each with an opportunity to recommend any changes it reasonably believes are necessary to continue to maintain

the Confidential Information disclosed by such other Party (or the Foundation, as applicable) to the Publishing Party in accordance with the requirements of this Agreement. The incorporation of such recommended changes shall not be unreasonably refused; and if a Party or the Foundation, as applicable, notifies ("**Publishing Notice**") the Publishing Party in writing, within [**] days after receipt of the copy of the proposed publication or presentation (or at least [**] days in the case of oral presentations), that such publication or presentation in its reasonable judgment (i) contains an Invention, solely or jointly conceived and/or reduced to practice by such Party, for which such Party reasonably desires to obtain patent protection or (ii) could be expected to have a material adverse effect on the commercial value of any Confidential Information disclosed by such Party or the Foundation to the Publishing Party, the Publishing Party shall prevent such publication or delay such publication for a mutually agreeable period of time. In the case of Inventions, a delay shall be for a period reasonably sufficient to permit the timely preparation and filing of a patent application(s) on such Invention, and in no event less than [**] days after the date of the Publishing Notice.

19.6 Commercial Considerations

Nothing in this Agreement shall prevent either Party from disclosing Confidential Information of the other Party or the Foundation, or the Parties, as applicable (i) to Regulatory Authorities, to the extent necessary to obtain or maintain INDs or Regulatory Approvals for any Product as permitted under this Agreement; (ii) to outside consultants, scientific advisory boards, and clinical investigators to the extent necessary to research, develop or commercialize any Compound or Product in accordance with this Agreement; provided that such Party, as applicable, shall obtain confidentiality obligations from such Third Parties substantially similar to the confidentiality provisions set forth in this Article 19; and (iii) to the extent necessary to Handle, defend and enforce Roche Patent Rights, PTC Patent Rights or Joint Patent Rights, in each of the foregoing cases, to the extent applicable to such Party's activities under this Agreement.

19.7 Interplay with SRA

This Article 19 shall replace Article 5 of the SRA commencing on the Effective Date. The foregoing shall not be interpreted as a waiver of any remedies available to PTC or Foundation as a result of any breach, prior to the Effective Date, by PTC or Foundation of its obligations under Article 5 of the SRA. At the end of the Agreement Term, this Article 19 shall continue to apply to Roche for an additional [**] years. If at the end of the Agreement Term, the SRA is still in effect, PTC and the Foundation shall thereafter be bound by Article 5 of the SRA. If at the end of the Agreement Term the SRA is no longer in effect, this Article 19 shall apply to PTC and the Foundation for an additional [**] years.

20. Term and Termination

20.1 Commencement and Term

This Agreement shall be binding upon the Parties as of the Execution Date to the extent permitted by the HSR Act, including without limitation the provisions of Sections 20.3.6 and 22.1, but shall not otherwise take effect until the Effective Date, and shall thereafter continue for the Agreement Term.

20.2 SRA Special Termination, SRA Reversion Notice and SRA Buy-Out Notice

So that Roche may effectuate the granting of licenses and rights as set forth in Section 2.2.2, PTC will immediately notify Roche about any SRA Special Termination or PTC's receipt of an SRA Reversion Notice or SRA Buy-Out Notice.

Notwithstanding anything in this Agreement to the contrary, PTC, following consultation with Roche, shall have the right to remedy any omission by Roche relating to Roche's obligations under this Agreement that PTC reasonably believes is likely, should such omission continue for at least [**] days, to trigger an SRA Special Termination, SRA Reversion Notice, SRA Buy-Out Notice or Foundation's right to terminate the SRA.

20.3 Termination

20.3.1 Termination for Breach

A Party ("**Non-Breaching Party**") shall have the right to terminate this Agreement in its entirety or on a country-by-country basis in the event the other Party ("**Breaching Party**") is in breach of any of its material obligations under this Agreement. The non-Breaching Party shall provide written notice to the Breaching Party, which notice shall identify the breach and the countries in which the Non-Breaching Party intends to have this Agreement terminate. The Breaching Party shall have a period of [**] days (or [**] days in the case of a payment breach or a breach by Roche that, if not cured by Roche, is likely to trigger an SRA Special Termination, SRA Reversion Notice, SRA Buy-Out Notice or Foundation's right to terminate the SRA) after such written notice is provided to cure such breach ("**Peremptory Notice Period**"). If such breach is not cured within the Peremptory Notice Period, this Agreement shall effectively terminate in such countries.

A Party shall have the right to terminate this Agreement if the other Party incurs an Insolvency Event; provided, however, in the case of any involuntary bankruptcy proceeding, such right to terminate shall only become effective if the Party that incurs the Insolvency Event consents to the involuntary bankruptcy or such proceeding is not dismissed within ninety (90) days after the filing thereof.

20.3.2 Termination by Roche for Change of Control of PTC

In the event of a Change of Control of PTC, Roche shall have the right, subject to Section 7.15, by written notice to PTC and Foundation given within the Election Period, to terminate this Agreement immediately, without prejudice to any of its other rights conferred on it by this Agreement or under applicable Law.

20.3.3 Termination by Roche Without a Cause

Roche shall have the right to terminate this Agreement at any time after the second anniversary of the Effective Date on a Product-by-Product and country-by-country basis (i) upon three (3) months' prior written notice before First Commercial Sale of the Product or (ii) upon nine (9) months' prior written

notice after the First Commercial Sale of the Product. The effective date of termination under this Section 20.3.3 shall be the date three (3) months or nine (9) months, as the case may be, after Roche provides such written notice to PTC.

20.3.4 Termination by PTC for Patent Challenge

If any member of the Roche Group (or Chugai with the acquiescence of Roche) challenges the validity, enforceability, patentability or scope of any claim included in any PTC Patent Right or Joint Patent Right, or supports, directly or indirectly, any such challenge (any of the foregoing, a "**Patent Challenge**"), PTC shall have the right to terminate this Agreement with respect to such challenged PTC Patent Right or Joint Patent Right upon thirty (30) days' written notice to Roche with respect to the PTC Patent Right or Joint Patent Right that is subject of the Patent Challenge. In addition, if such Patent Challenge is terminated during such thirty (30) day period, then PTC shall not have the right to terminate this Agreement in respect of such Patent Challenge; provided, however, that Roche shall reimburse PTC for all costs and expenses, including without limitation attorneys' fees, incurred by PTC in defending such Patent Challenge, and shall pay all such reimbursement amounts within [**] days after receipt of an invoice from PTC therefor.

20.3.5 Termination by PTC for Post-Change of Control Material Change

PTC shall have the right to terminate this Agreement as set forth in Section 22.2.

20.3.6 Termination for Delay in Effective Date

Either Party may terminate this Agreement effective upon notice to the other Party if the HSR Clearance Date shall not have occurred on or prior to the date ninety (90) days after the Parties make their respective HSR Act filings pursuant to Section 22.1. If this Agreement is terminated pursuant to this Section 20.3.6, this Agreement, including without limitation Section 20.5, shall terminate in its entirety and shall be void and of no force or effect.

20.4 Consequences of Termination

20.4.1 Termination by Roche for Convenience; Termination by PTC for Roche Breach, Insolvency Event, Patent Challenge or Post-Change of Control Material Change

If this Agreement is terminated by Roche pursuant to Section 20.3.3 (Termination Without a Cause), or by PTC pursuant to Section 20.3.1 (Termination for Breach), Section 20.3.4 (Termination for Patent Challenge) or Section 20.3.5 (Termination for Post-Change of Control Material Change), then, in addition to the consequences set forth in Section 20.3.4 with respect to Patent Challenge and the consequences set forth in Sections 20.4.3 and 20.5, and subject to Section 20.4.1.8:

20.4.1.1 Termination of Licenses. The licenses granted by PTC to Roche pursuant to Section 3.1 shall terminate;

20.4.1.2 Regulatory Matters. Promptly following receipt of PTC's written notice that it intends to continue development and commercialization of Compounds and Products, Roche shall transfer and assign to PTC ownership of:

(i) all regulatory filings and Regulatory Approvals in Roche's or its Affiliates' possession or control relating to all Compounds and Products; and

(ii) any regulatory dossiers containing information necessary or useful to Roche in connection with its regulatory filings for all Products, including, but not limited to clinical trial dossiers, regulatory correspondence, Regulatory Authority meeting minutes and study reports from completed non-clinical studies. For all completed study reports, Roche shall provide necessary documentation to confirm data reliability, as required by Article 43 of the Japanese Pharmaceutical Affairs Law Enforcement Regulations and related notifications, including, but not limited to original author signatures, raw data lists, GLP and GCP compliance information. All documentation is to be provided in English.

20.4.1.3 Preclinical and Clinical Matters. Promptly following receipt of PTC's written notice that it intends to continue development and commercialization of Compounds and Products, Roche shall assign to PTC its entire right, title, and interest in and to all preclinical and clinical data, including but not limited to pharmacology and biology data, in Roche's or its Affiliates' possession or control relating to and to the extent necessary for PTC to continue the research, development or commercialization of Compounds and Products;

20.4.1.4 Manufacturing Matters. Promptly following receipt of PTC's written notice that it intends to continue development and commercialization of Compounds and Products, Roche shall:

(i) assign each manufacturing agreement entered into by any member of the Roche Group that is specific to Compounds or Products to PTC, if

such agreement is then in effect and such assignment is permitted under such agreement or by the applicable counterparty;

(ii) cooperate with PTC to transfer manufacturing documents and materials that are used (at the time of the termination) by any member of the Roche Group or Third Party contract manufacturers in the manufacture of Compounds and Products, to the extent such manufacturing documents and materials are not obtained by PTC pursuant to the assignment of agreements pursuant to Section 20.4.1.4(i);

(iii) for a period of up to [**] months after the effective date of termination, subject to the then-existing obligations to Third Parties, cooperate with PTC to transfer to PTC, in a manner consistent with the guidelines set forth in Appendix 8.2.2, manufacturing technologies and all associated Know-How that are used (at the time of the termination) by any member of the Roche Group or Third Party contract manufacturers in the manufacture of Compounds and Products including, without limitation, providing, following reasonable advance request by PTC, [**] of up to [**] days duration of one Roche full-time equivalent to provide technical assistance in transferring technology required for the manufacture of a Compound at a PTC, PTC Affiliate, or subcontractor manufacturing facility; provided that PTC shall reimburse Roche for Roche's reasonable out-of-

pocket expenses to provide such requested assistance, to the extent such manufacturing technologies are not obtained by PTC pursuant to Section 20.4.1.4(i);

(iv) sell the Roche Group's then-existing inventory of Compounds and Products to PTC, at Roche's FBMC plus [**] percent ([**]%); and

(v) if this Agreement is terminated after Initiation of a Pivotal Trial of a Product, use Commercially Reasonable Efforts to manufacture or have manufactured and supply PTC's requirements of such Product for a period of up to [**] months after the effective date of termination, at Roche's FBMC plus [**] percent ([**]%).

20.4.1.5 License Grants to PTC. Subject to the then-existing obligations to Third Parties, Roche hereby grants to PTC:

(a) a non-exclusive, perpetual right and license under Roche's interest in the Roche Background Patent Rights and Roche Know-How; and

(b) an exclusive, perpetual right and license under Roche's interest in the Roche Product Patent Rights and Roche's interest in the Joint Patent Rights and Joint Know-How;

in each case ((a) and (b)), with the right to grant sublicenses, to develop, have developed, register, have registered, use, have used, make, have made, import, have imported, export, have exported, market, have marketed, distribute, have distributed, sell and have sold Compounds and Products then being developed or commercialized by Roche in the Field in the Territory.

20.4.1.6 Prosecution and Enforcement. The provisions of Sections 15.5 (Prosecution of Patent Rights Claiming Joint Inventions) and 15.8 (Infringement) shall remain in effect with respect to the Joint Patent Rights and Joint Know-How licensed to PTC under Section 20.4.1.5;

20.4.1.7 Assignment of Trademark. Promptly following receipt of PTC's written notice that it intends to continue development and commercialization of Compounds and Products, Roche shall assign to PTC Roche's and its Affiliates' entire right, title and interest in, to and under any trademark used by any member of the Roche Group exclusively in connection with the commercialization of a Product, it being understood that such assignment shall not include the Roche name or trademark for the Roche company itself; and

20.4.1.8 Remaining Products, Countries and Patent Rights. If PTC terminates this Agreement pursuant to Section 20.3.1 with respect to a specific country(ies), or pursuant to Section 20.3.4 with respect to a specific PTC Patent Right or Joint Patent Right, or Roche terminates this Agreement pursuant to Section 20.3.3 with respect to a specific country(ies) or Product(s), then this Agreement shall remain in full force and effect for the non-terminated country(ies), Patent Right(s) and/or Product(s) (as the case may be), and all of the consequences set forth in this Section 20.4.1 shall apply solely with respect to the terminated country(ies), Patent Right(s) and/or Product(s).

20.4.1.9 Assumption by Foundation. If Roche has not received written notice from PTC pursuant to Section 20.4.1.2, 20.4.1.3, 20.4.1.4, or 20.4.1.7 by [**] days after the effective date of termination of this Agreement, then Roche shall promptly notify Foundation and Foundation shall have the right, upon notice to Roche, to require Roche to provide to Foundation or its designee all items and actions that Roche would have been obligated to provide to PTC if PTC had provided such notice.

20.4.2 Termination by Roche for Breach by PTC

Upon any termination by Roche for breach by PTC, the licenses granted to Roche pursuant to Section 3.1 shall continue solely with respect to Compounds and Products, subject to Roche's continued compliance with Roche's payment and other obligations under Articles 11, 12, 13 and 14 and further subject to Section 2.2.2.

20.4.3 Other Obligations

20.4.3.1 Obligations Related to Ongoing Activities

From the date of notice of termination until the effective date of termination, Roche shall continue activities, including but not limited to preparatory activities, ongoing as of the date of notice of termination and shall be responsible for all of its uncancelable obligations. However, Roche shall not be obliged to initiate any new activities not ongoing at the date of notice of termination.

In any case, after the effective date of termination, Roche shall not have any obligation to perform and/or complete any activities or to make any payments for performing or completing any activities under this Agreement.

Notwithstanding the foregoing, in case of termination by PTC under Section 20.3.1, 20.3.2, 20.3.4 or 20.3.5, or by Roche under Section 20.3.3, upon the request of PTC or Foundation, Roche shall complete any Clinical Studies related to the Product that are being conducted under its IND for the Product and are ongoing as of the effective date of termination; provided, however, that Roche may agree but shall have no obligation to recruit or enroll any additional patients after the date of termination.

20.4.3.2 Royalty and Payment Obligations

(a) Termination of this Agreement by a Party, for any reason, shall not release Roche from any obligation to pay royalties or make any payments to PTC that are due and payable prior to the effective date of termination.

(b) In consideration for the value added by Roche with respect to Compounds and Products and for the licenses granted in Section 20.4.1.5, PTC shall pay to Roche in the event this Agreement is terminated for any reason other than a material breach by Roche, royalties during

the applicable Royalty Term on Products as follows:

Stage of Product at time of termination	Royalty on Product revenues as a percentage of Net Sales
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

(c) If PTC receives any payment from Foundation as the result of Foundation's right to commercialize Products, then, in lieu of royalties under Section 20.4.3.2(b), PTC shall pay to Roche [**] percent ([**]%) of any such payment received from Foundation; provided, however, that Roche shall not be entitled to any payment under this Section 20.4.3.2 if Foundation's right to commercialize Products was triggered by Roche's breach of any of its obligations under this Agreement.

(d) All amounts payable from PTC to Roche under this Section 20.4.3.2 shall be subject to the provisions of Sections 11.5.2, 11.5.3, 11.5.4, 11.5.5, 11.5.6 and Articles 12, 13 and 14, and any defined terms referenced therein, *mutatis mutandis*.

(e) For clarity, nothing in this Section 20.4.3.2 shall modify Foundation's rights or PTC's obligations under the SRA or trigger any payment obligation of Foundation to Roche.

20.5 Survival

Article 1 (Definitions), Section 2.2.2 (Termination and Grant-back Provisions), Section 3.4 (Rights Retained by the Parties), Article 12 (Accounting and Reporting), Article 13 (Taxes), Article 14 (Auditing), Section 15.1 (Ownership of Inventions), Article 17 (Indemnification), Article 18 (Disclaimer), Article 19 (Obligation Not to Disclose Confidential Information), Section 20.4 (Consequences of Termination), Section 20.5 (Survival), Article 22 (Miscellaneous) shall survive any expiration or termination of this Agreement for any reason, other than a termination pursuant to Section 20.3.6 (Termination for Delay in Effective Date).

21. Bankruptcy

All licenses (and to the extent applicable rights) granted under or pursuant to this Agreement by PTC to Roche are, and shall otherwise be deemed to be, for purposes of Section 365(n) of Title 11, US Code (the "**Bankruptcy Code**") licenses of rights to "intellectual property" as defined under Section 101(60) of the Bankruptcy Code. Unless Roche elects to terminate this Agreement, the Parties agree that Roche, as a licensee or sub-licensee of such rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the Bankruptcy Code, subject to the continued performance of its obligations under this Agreement.

22. Miscellaneous

22.1 HSR Act

If in the reasonable opinion of counsel to Roche or PTC it is required that the Parties make a filing under the HSR Act, Roche and PTC will promptly following the Execution Date (i) take all actions necessary to make any filing required under the HSR Act and (ii) reply at the earliest possible date with any requests for information received from the United States Federal Trade Commission ("**FTC**") or Antitrust Division of the United States Department of Justice ("**DOJ**") pursuant to the HSR Act. The Parties will, to the extent reasonably practicable, consult with one another prior to making any filings, responses to inquiries or other contacts with the FTC or DOJ concerning the transactions contemplated by this Agreement and will use Commercially Reasonable Efforts to obtain any clearances related to this Agreement that are necessary under the HSR Act. Each Party will be responsible for its own costs in connection with such filing, except that Roche will be solely responsible for the applicable filing fees. Roche shall provide PTC notice of achievement of the HSR Clearance Date on the HSR Clearance Date.

22.2 Change of Control of Roche

Following a Change of Control of Roche, it is the expectation of the Parties that the research, development and commercialization of Products will continue in substantially the same manner as immediately prior to the occurrence of such Change of Control, including without limitation as reflected in the then applicable Research Plan, SRA Development Plan and New Product Development Plan. Within [**] days following the closing of a Change of Control, Roche shall provide PTC and the Foundation written certification from Roche and Roche's acquirer that Roche will continue to perform all of its obligations under this Agreement. In furtherance of the foregoing, with respect to each Product the following provisions shall apply:

(a) The Research Plan, SRA Development Plan and the New Product Development Plan in effect as of the occurrence of each Change of Control for such Product shall remain in effect for the remaining period covered by such plans, unless otherwise agreed by the Parties.

(b) If PTC believes that a Post-Change of Control Material Change (as defined below) has occurred with respect to such Product, PTC shall provide written notice of such belief to Roche and the Foundation. Within [**] days after receipt of such notice, Roche shall respond in writing to PTC, and the Parties shall meet within [**] Business Days thereafter to discuss the issues raised in the notice from PTC and the response from Roche. The Parties shall attempt in good faith to reach consensus on whether a Post-Change of Control Material Change has occurred with respect to such Product in accordance with Section 22.6.

In the event that either (i) the Parties agree that a Post-Change of Control Material Change has occurred with respect to such Product, or (ii) a final judicial determination is made in accordance with Section 22.6 that a Post-Change of Control Material Change has occurred with respect to such Product, then PTC shall have the right to terminate this Agreement effective upon written notice to Roche within [**] days after the date of such agreement or determination.

As used in this Section 22.2, the term "**Post-Change of Control Material Change**" means, with respect to a Product, (A) there has been a material adverse impact on the market for such Product as a result of specific actions or omissions by Roche or its successor that do not comply, or are inconsistent, with the then applicable Research Plan, SRA Development Plan, or New Product Development Plan, or (B) specific actions or omissions by Roche or its successor have triggered the availability of a right on the part of Foundation to cause an SRA Special Termination, to obtain an SRA Reversionary License and/or to exercise the SRA Buy-Out Right, in each case pursuant to the procedures set forth in the SRA.

22.3 Standstill

Until [**] years following an initial public offering of PTC, neither Roche nor any of its Affiliates shall make an unsolicited offer to acquire PTC or PTC securities representing in excess of [**] ownership of PTC. Irrespective of the foregoing, if PTC either determines to pursue a sale of PTC, or becomes the subject of an unsolicited bid from a party other than Roche or a

Roche Affiliate, then the standstill shall be suspended during the period in which such merger and acquisition activities or sale process are active.

22.4 Hub

As used in this Agreement, "**Hub**" shall mean a license, option, joint venture, collaboration, sale, assignment or other strategic transaction, vehicle or mechanism under which Roche develops and commercializes Products and/or the majority of its products for orphan and/or rare diseases, including without limitation any transfer of or option to Roche's rights under this Agreement to research, develop, make, have made, use, sell or import any Product in the Field, but excluding any outsourcing contract for research or development, work, distribution, co-marketing, manufacturing or other similar commercial arrangement entered into with a Third Party under which Roche remains primarily responsible for development and commercialization of Products. The Parties shall consider in good faith the possibility of Roche and PTC creating a Hub.

22.5 Governing Law

This Agreement shall be governed by and construed in accordance with the Laws of the state of Delaware, US, without reference to its conflict of laws principles; provided that, with respect to matters involving enforcement of intellectual property rights, the Laws of the applicable country shall apply. The provisions of the United Nations Convention on Contracts for the International Sale of Goods shall not apply to this Agreement or any subject matter hereof. For clarity, to the extent that this Agreement amends the SRA, such amended provision of the SRA shall remain subject to the governing law and dispute resolution provisions of the SRA.

22.6 Disputes

Unless otherwise set forth in this Agreement, any dispute in connection with this Agreement shall be referred to the respective executive officers of the Parties and the Foundation designated below or their designees, for good faith negotiations attempting to resolve the dispute. The designated executive officers are as follows:

For PTC:	Chief Executive Officer
For Roche:	Head of Partnering
For Foundation:	President

Should the good faith negotiations fail to agree within [**] days after a dispute has first arisen, either Party or the Foundation, as applicable, may seek to resolve such dispute in any court having jurisdiction over whichever of PTC, Roche or the Foundation are in dispute.

22.7 Equitable Relief

Each Party and the Foundation acknowledges and agrees that the other Party and the Foundation, or the Parties, as applicable, may be damaged irreparably in the event any of the provisions of Section 3.5 or Article 19 are not performed in accordance with their specific terms or otherwise are breached. Accordingly, notwithstanding Sections 7.7.3, 7.9.3 and 22.6, each

Party and the Foundation agrees that the other Party or the Foundation, or either Party, as applicable, shall be entitled to seek an injunction or other equitable relief to prevent breaches of such provisions and to enforce specifically such provisions in any action instituted in any court having jurisdiction over the applicable Party(-ies) (and the Foundation, as applicable) and the matter, in addition to any other remedy to which it may be entitled, at law or in equity.

22.8 Assignment

Neither Party may assign its rights or obligations under this Agreement absent the prior written consent of the other Party, except to any of its Affiliates or in the context of a merger, acquisition, sale or other transaction involving all or substantially all of the assets of the Party seeking to assign, in which case such Party in its sole discretion may assign its rights and obligations under this Agreement. Foundation may assign its rights or obligations under this Agreement to any Person to whom its assigns its rights and obligations under the SRA. Any permitted assignment shall be binding on the successors of the assigning Party.

22.9 Independent Contractor

No employee or representative of either Party or the Foundation shall have any authority to bind or obligate the other Party or the Foundation, or either of the Parties, as applicable, for any sum or in any manner whatsoever or to create or impose any contractual or other liability on the other Party or the Foundation, or either of the Parties, as applicable, without the prior written approval of the other Party, the Foundation, the Party, as applicable,. For all purposes, and notwithstanding any other provision of this Agreement to the contrary, PTC's legal relationship to Roche under this Agreement shall be that of independent contractor.

22.10 Unenforceable Provisions and Severability

If any provision of this Agreement is held unenforceable by a court or tribunal of competent jurisdiction because it is invalid or conflicts with any Law of any relevant jurisdiction, the validity of the remaining provisions shall not be affected. In such event, the Parties and, with respect to any Foundation Provision or any other provision of this Agreement pursuant to which a Party owes an obligation to the Foundation or from which the Foundation would benefit, the Parties and the Foundation, shall negotiate a substitute provision that, to the extent possible, accomplishes the original business purpose.

22.11 Waiver

The failure by either Party or the Foundation to require strict performance and/or observance of any obligation, term, provision or condition under this Agreement will neither constitute a waiver thereof nor affect in any way the right of the respective Party or the Foundation to require such performance and/or observance. The waiver by either Party or the Foundation of a breach of any obligation, term, provision or condition hereunder shall not constitute a waiver of any subsequent breach thereof or of any other obligation, term, provision or condition.

22.12 Appendices

All Appendices to this Agreement shall form an integral part to this Agreement.

22.13 Entire Understanding

This Agreement contains the entire understanding among the Parties and the Foundation with respect to the within subject matter and supersedes any and all prior agreements, understandings and arrangements, whether written or oral, except for the SRA.

22.14 Amendments

No amendments of the terms and conditions of this Agreement shall be binding upon the Parties unless in writing and signed by each of the Parties; provided, however, that any amendment of any Foundation Provision or any other provision of this Agreement pursuant to which a Party owes an obligation to the Foundation or from which the Foundation would benefit shall not be binding upon the Parties or the Foundation unless also signed by the Foundation.

22.15 Invoices

All invoices that are required or permitted hereunder shall be in writing and sent by PTC or the Foundation, as applicable, to Roche at the following address or other address as Roche may later provide in writing:

Hoffmann-La Roche Inc.
Accounts Payable
340 Kingsland Street
Nutley, New Jersey 07110

22.16 Notice

All notices that are required or permitted hereunder shall be in writing and sufficient if delivered personally, sent by facsimile (and promptly confirmed by personal delivery, registered or certified mail or overnight courier), sent by nationally recognized overnight courier or sent by registered or certified mail, postage prepaid, return receipt requested, addressed as follows:

if to PTC, to:

PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, New Jersey 07080
U.S.A.
Attn: Legal Department
Facsimile No.: (908) 222-1128

With an email copy to: legal@ptcbio.com

And:

WilmerHale
60 State Street
Boston, Massachusetts 02109
U.S.A.
Attn: Steven D. Singer, Esq.
Facsimile No.: (617) 526-5000

if to Roche, to:

F. Hoffmann-La Roche Ltd
Grenzacherstrasse 124
4070 Basel
Switzerland
Attn: Legal Department
Facsimile No.: +41 61 688 13 96

And:

Hoffmann-La Roche Inc.
340 Kingsland Street
Nutley, New Jersey 07110

U.S.A.
Attn. Corporate Secretary
Facsimile No.: +1 973 235-3500

if to the Foundation, to: Spinal Muscular Atrophy Foundation
888 Seventh Avenue, Suite 400
New York, New York 10019
U.S.A.
Attn: President
Facsimile No: 212-247-3079

And: Cooley LLP
4401 Eastgate Mall
San Diego, CA 92121
U.S.A.
Attn: Matthew Browne, Esq.
Facsimile No: 858-550-6420

or to such other address as the notice recipient may have furnished to the notice provider in writing in accordance herewith.

Each Party shall provide a copy of any notice sent pursuant to this Agreement to Foundation in accordance with this Section 22.16.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties and the Foundation have entered into this Agreement as of the Effective Date.

PTC Therapeutics, Inc.

By: /s/ Stuart Peltz

Name: Stuart Peltz

Title: President and CEO

F. Hoffmann-La Roche Ltd

By: /s/ Dan Zabrowski

Name: Dan Zabrowski

Title: Global Head Roche Partnering

By: /s/ Stefan Arnold

Name: Stefan Arnold

Title: Head Legal Phama

Hoffmann-La Roche Inc.

By: /s/ Joseph S. McCracken

Name: Joseph S. McCracken

Title: Vice President

Spinal Muscular Atrophy Foundation (solely with respect to the Foundation Provisions)

By: /s/ Florence (Loren) Eng

Name: Florence (Loren) Eng

Title: President

11/22/11

Appendix 1.18

Development Candidate Criteria

Property	Goal	Assay Description	Notes
Activity in SMN2 RNA assay			

[**]	[**]	[**]	
[**]	[**]	[**]	
Activity in SMN protein assays			
[**]	[**]	[**]	
[**]	[**]	[**]	
Selectivity			
[**]	**[**]**	**[**]**	
Metabolism			
[**]	[**]	[**]	
[**]	[**]	[**]	
Pharmacokinetics			
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]		
Efficacy [**]			
[**]	[**]	[**]	[**]
[**]	[**]	[**]	
[**]	[**]	[**]	
Efficacy [**]			
[**]	[**]	[**]	[**]
Safety			
[**]	[**]	[**]	
[**]	[**]	[**]	
Profiling and ancillary pharmacology			
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	

Property	Goal	Assay Description	Notes
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	[**]
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	
[**]	[**]	[**]	
Compound properties			
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
[**]	[**]		
Others			
[**]	[**]		
[**]	[**]		
[**]	[**]	[**]	[**]

Appendix 1.63

PTC Base Patent Rights

[**]

Appendix 1.69

Research Plan

PTC and Roche Research Plan

Overview

The goal of the collaboration is to identify potential Development Candidates that increase the expression of SMN protein by correcting the alternative splicing of SMN2 pre-mRNA. The Parties will perform medicinal chemistry, including [**].

Table 1: Proposed Research Activities and indicative FTEs (to be confirmed at the first JSC):

AREA	FTE	ACTIVITIES
Medicinal Chemistry	[**]	[**]
Biology	[**]	[**]
Pharmacology	[**]	[**]
Formulation	[**]	[**]
Bioanalytical/Metabolism	[**]	[**]
Cheminformatics	[**]	[**]
Alliance Management	[**]	[**]
Total	[**]	

Other Potential Activities:

Synthetic Chemistry

[**]

Biology, Pharmacology, Metabolism and other studies

[**]

Bioanalytical and Formulation

[**]

Contract Research Organization Activities:

[**]

Figure 1

SMA Screening Tier (studies performed at PTC)

[**]

Appendix 1.72

Patent Rights Excluded from Roche Background Patent Rights

[**] Patent Rights, which means (a) [**], and any and all patents issuing from divisionals, continuations, or continuations-in part of any application from which [**] claims priority, as well as reissues, reexaminations, extensions, and foreign patent counterparts, including inventors certificates, of any of the foregoing, and including any related supplemental protection certificates; and (b) [**], and any and all patents issuing from divisionals, continuations, or continuations-in-part of any application from which [**] claims priority, as well as reissues, reexaminations, extensions, and foreign patent counterparts, including inventors certificates, of any of the foregoing, and including any related supplemental protection certificates.

[**] Patent Rights, which means any of the U.S. patents listed below and any and all patents issuing from divisionals, continuations or continuations-in-part, and any reissues, reexaminations or extensions, of these patents or of any application from which these U.S. patents claim priority, as well as foreign counterparts, including inventors certificates, of the foregoing, and including any related supplemental protection certificates:

[**]

[**] Patent Rights, which means any of the U.S. patents/patent application listed below and any and all patents issuing from divisionals, continuations or continuations-in-part, and any reissues, reexaminations or extensions, of these patents or of any application from which these U.S. patents claim priority, as well as foreign counterparts, including inventors certificates, of the foregoing, and including any related supplemental protection certificates:

[**]

[**] Patents, which means the following U.S. patent and any and all divisionals, continuations, continuations-in-part of any application from which these U.S. patents claim priority, including reissues, reexaminations or extensions of these patents and foreign counterparts and supplementary protection certificates of the foregoing:

[**]

Appendix 1.81

Sponsored Research Agreement

Incorporated by reference to Exhibit 10.15 of the Company's Registration Statement on Form S-1

Appendix 1.88

Transitional Research Plan

The goal of the proposed research is to characterize potential Development Candidates that increase the expression of SMN protein by correcting the alternative splicing of SMN2 pre-mRNA. The SMA program at PTC is currently at the [**] stage. PTC will conduct research at the level outlined below from the signing of the Agreement until approval of the Research Plan by the Joint Steering Committee (JSC). PTC will [**].

PTC activities from Execution Date to Research Plan approval:

AREA	FTE	ACTIVITIES
Medicinal Chemistry	[**]	[**]
Biology	[**]	[**]
Pharmacology	[**]	[**]
Formulation	[**]	[**]
Bioanalytical/Metabolism	[**]	[**]
Total	[**]	

Appendix 8.2.2

Technology Transfer Guidelines

Responsibility
[**]

Manufacturing Instructions
[**]

Evaluate Changes to Manufacturing Processes
[**]

Transfer Protocol
[**]

Storage / Transportation Instructions
[**]

Environmental Controls Cleanliness Zones
[**]

Hygiene Instructions / occupational health instructions
[**]

Appendix 11.1

Invoice for Research Recognition Payment

PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080
(908) 222-7000
www.ptcbio.com

Date	Inv. #
November 23, 2011	Roc11172011

Bill To:	Ship To:
Hoffmann-La Roche Inc. Accounts Payable 340 Kingsland Street Nutley, NJ 07110	Hoffmann-La Roche Inc. Accounts Payable 340 Kingsland Street Nutley, NJ 07110

PO#	Terms	Ship Date	Carrier	FOB
	Per Contract			
Quantity	**Product**	**Description**	**Unit Price**	**Amount**
1	Per Contract	Research Recognition Payment as per Section 11.1 of the License and Collaboration Agreement		$ 30,000,000.00
			Amount Due	**$ 30,000,000.00**

Bank and VAT details of PTC Therapeutics, Inc:	
Account holder's name:	PTC Therapeutics, Inc.
Account no;	[**]
IBAN (for Europe) / ABA#:	021200025
Swift #:	PNBPUS33
Bank Name:	Wachovia Bank NA
Bank Address:	MAC N 2684-020 120 Mountain View Blvd, Suite 200 Basking Ridge, NJ 07920
Registered office of bank:	USA

Appendix 19.4

Form of Press Release

Joint Roche Partnering-PTC-SMA Foundation Press release

Basel, Switzerland, South Plainfield, NJ, USA and New York, NY, USA — November 29, 2011

Roche signs agreement with PTC Therapeutics to advance treatment for Spinal Muscular Atrophy (SMA)
Collaboration offers new hope for a potential treatment for the leading genetic cause of death in infants and toddlers

Roche (SIX: RO, ROG; OTCQX: RHHBY), PTC Therapeutics, Inc. (PTC) and the SMA Foundation, announced today a licensing agreement for PTC's Spinal Muscular Atrophy (SMA) programme. SMA is a genetic neuromuscular disorder that causes muscle weakness. One in every 10,000 children born is affected with the disorder, which currently has no effective treatment.

PTC Therapeutics' programme has been developed in partnership with the SMA Foundation, which will remain active in the collaboration. The SMA Foundation was established in 2003 by Loren Eng and Dinakar Singh to accelerate the development of a treatment for SMA.

SMA is caused by a missing or defective SMN1 gene, which results in reduced levels of the survival motor neuron (SMN) protein. The compounds in PTC's research treat the underlying cause of the disorder and demonstrate increases in SMN levels in nervous system, muscles and other tissues in SMA models. SMA is

a rare disorder and could be eligible for orphan status by regulatory authorities, thereby potentially reducing the time needed for a drug to reach patients.

Under the terms of the agreement, Roche gains an exclusive worldwide license to PTC's SMA programme, which includes three compounds currently in preclinical development, as well as potential back-up compounds. PTC receives USD30 million as an upfront payment, up to USD460 million upon successful completion of certain development and commercialization milestones, and up to double-digit royalties on commercial sales. Development will be overseen by a joint steering committee comprised of members from Roche, PTC and the SMA Foundation.

"This collaboration brings us one step closer to developing a treatment for a condition that has a profound effect on the lives of many thousands of children and their parents worldwide," said Dinakar Singh,

Chairman of the SMA Foundation. "We are very optimistic that, by building on the pioneering efforts of PTC Therapeutics, Roche can help us realise what we have been working so hard to achieve."

Luca Santarelli, Global Head of Roche Neuroscience, said: "We found the science behind this programme very compelling, with the potential to help treat a currently incurable condition. This is the essence of Roche's entire strategy, focused on solid science and high unmet clinical need, and these compounds bolster our rich pipeline in Central Nervous System diseases. As an established partner of Roche, we already have experience with PTC's scientific approach. Together with the involvement of the SMA Foundation, we now have the opportunity to make a significant impact in the treatment of SMA."

"Having been a partner with Roche for several years, we have every confidence that the combination of our own expertise and Roche's considerable capabilities in clinical development, biomarkers and diagnostics will help us maximize the potential for this programme," commented Stuart W. Peltz, Ph.D., President and CEO of PTC. "We are delighted that the SMA Foundation continues to be an active participant in the collaboration as we share a strong commitment to advancing this innovative potential treatment as rapidly as possible."

Roche and PTC announced their first collaboration in September of 2009 for the development of orally bioavailable small molecules utilizing PTC's technology called Gene Expression Modulation by Small-molecules (GEMS™). The SMA programme has been developed by PTC utilizing a different scientific approach than GEMS, called alternative splicing.

About the SMA Foundation

Founded in 2003, the Spinal Muscular Atrophy Foundation is a nonprofit organization dedicated to accelerating progress towards a treatment for Spinal Muscular Atrophy through targeted funding of clinical research and novel drug development efforts. Since its inception, the Foundation has awarded over $100 million for SMA research. In addition, the Foundation is committed to raising awareness and generating support for increased research efforts in SMA among the leaders of industry and government. For more information, visit the SMA Foundation website at www.smafoundation.org.

About PTC Therapeutics, Inc.

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central

importance to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare genetic disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and that have served as the basis for collaborations with leading biopharmaceutical companies such as AstraZeneca, Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's website at www.ptcbio.com.

About Roche

Headquartered in Basel, Switzerland, Roche is a leader in research-focused healthcare with combined strengths in pharmaceuticals and diagnostics. Roche is the world's largest biotech company with truly differentiated medicines in oncology, virology, inflammation, metabolism and CNS. Roche is also the world leader in in-vitro diagnostics, tissue-based cancer diagnostics and a pioneer in diabetes management. Roche's personalised healthcare strategy aims at providing medicines and diagnostic tools that enable tangible improvements in the health, quality of life and survival of patients. In 2010, Roche had over 80,000 employees worldwide and invested over 9 billion Swiss francs in R&D. The Group posted sales of 47.5 billion Swiss francs. Genentech, United States, is a wholly owned member of the Roche Group. Roche has a majority stake in Chugai Pharmaceutical, Japan. For more information: www.roche.com

For more information please contact

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Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

SPONSORED RESEARCH AGREEMENT

THIS SPONSORED RESEARCH AGREEMENT (the "***Agreement***") is entered into as of June 1, 2006 (the "***Effective Date***"), by and between **SPINAL MUSCULAR ATROPHY FOUNDATION** (the "***Foundation***"), having its principal place of business located at 1776 Broadway, 22nd Floor, New York, New York, 10019, and **PTC THERAPEUTICS, INC.** ("***PTC***" or "***Company***"), having its principal place of business located at 100 Corporate Court, South Plainfield, New Jersey, 07080.

RECITALS

WHEREAS, Company is focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms;

WHEREAS, the Foundation is dedicated to accelerating the development of a treatment or cure for spinal muscular atrophy;

WHEREAS, the Foundation wishes to sponsor, and Company wishes to perform, research focused on small molecule therapeutics for spinal muscular atrophy ("***SMA***"), and possibly to further develop and commercialize such therapeutics, subject to the terms and conditions of this Agreement, including the Research Plan attached hereto as **Exhibit A**;

WHEREAS, it is the intent of the Foundation and Company to disseminate the results of the Research (as defined below) to other investigators in the spinal muscular atrophy research community and to medical professionals treating spinal muscular atrophy patients, consistent with the overall goal of commercializing therapeutics for SMA; and

WHEREAS, it is the further intent of the Foundation and Company that patents and other intellectual property developed by Company as a result of the Research shall be retained by Company, but that a mechanism be provided for transfer of rights to the patents and other intellectual property developed by Company as a result of the Research and relating to a particular Research Project (as defined below) to the Foundation if Company elects not to pursue commercial development of any drug candidates identified during such Research Project.

NOW, THEREFORE, in consideration of the foregoing and the mutual covenants and premises contained in this Agreement, the parties hereto agree as follows:

1. DEFINITIONS.

1.1 "Additional Payments" shall mean all amounts actually paid to Company pursuant to Section 4.2.

1.2 "Affiliate" shall mean any corporation or other entity that controls, is controlled by, or is under common control with, a party. A corporation or other entity shall be regarded as in control of another corporation or entity if it owns or directly or indirectly controls more than 50% of the voting securities or other ownership interest of the other corporation or entity, or if it possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of the corporation or other entity.

1.3 "Available Product" shall mean a human therapeutic product that (a) has previously received final approval from the FDA for marketing in the United States and (b) is suitable for administration to patients in its currently marketed formulation for the treatment of spinal muscular atrophy.

1.4 "Company Base IP" shall mean any new and useful composition of matter, process, product by process, machine or manufacture, know-how, discovery, improvement, Patent, or other intellectual property ("**IP**") or any new and useful improvement thereof, whether or not patentable, which (i) is discovered, conceived, developed or first reduced to practice by or on behalf of Company as of or prior to the Effective Date, (ii) is an improvement to any IP discovered, conceived, developed or first reduced to practice by or on behalf of Company as of or prior to the Effective Date, regardless of when such improvement is discovered, conceived, developed or first reduced to practice, or (iii) is discovered, conceived, developed or first reduced to practice by, or otherwise comes under the Control of, Company during the Research Term and does not constitute Data or a Research Invention.

1.5 "Company Clinical Trial" shall have the meaning provided in Section 3.4.

1.6 "Company Know-How" shall mean Information that: (a) is developed or acquired by or on behalf of Company in the course of performing the Research; and/or (b) is otherwise Controlled by Company and is directed to any Drug Target, Hit, Lead Candidate, Drug Candidate or Product first identified or synthesized in the conduct of the Research, formulations of any of the foregoing, and/or processing technology with respect thereto; *provided, however,* that the Company Know-How excludes the Company Patents and the Company Base IP.

1.7 "Company Patents" shall mean Patents that: (a) claim Information developed or acquired by or on behalf of Company in the course of performing the Research; and/or (b) are otherwise Controlled by Company and claim any Drug Target, Hit, Lead Candidate, Drug Candidate or Product first identified or synthesized in the course of the Research, formulations of any of the foregoing, and/or processing technology with respect thereto; *provided, however,* that the Company Patents exclude the Company Base IP and the Company Know-How.

1.8 "Company Technology" shall mean Company Know-How and Company Patents.

1.9 "Confidential Information" shall mean any confidential or proprietary information of a party, including, without limitation, information relating to any compound, product specifications, chemical structures, data, know-how, formulations, research project, work in process, future development, scientific, engineering, manufacturing, marketing, business plan, financial or personnel matter relating to such party, its present or future products, sales, suppliers, customers, employees, investors or business, whether in oral, written, graphic or electronic form, subject to the provisions of Section 5.2 hereof. Without limiting the generality of the foregoing, the terms of this Agreement shall be deemed the Confidential Information of both parties, subject to Section 5.5.

1.10 "Control" shall mean, with respect to any Information, Patent or other intellectual property right, possession by a party of the ability (whether by ownership, license or

otherwise) to grant access, a license or a sublicense to such Information, Patent or other intellectual property right without (a) violating the rights of any Third Party or the terms of any agreement or other arrangement with any Third Party, and (b) incurring any additional cost or royalty obligation to such Third Party based on the granting of such access, license or sublicense.

1.11 "Data" shall have the meaning provided in Section 6.1(a).

1.12 "Drug Candidate" shall mean a Hit, Lead Candidate or any metabolite, prodrug, solvate (including without limitation any hydrate), ester, salt, stereoisomer, racemate, tautomer or polymorph of such Hit or Lead Candidate that is first synthesized or identified in the conduct of the Research and that exhibits desired levels of activity against the applicable Drug Target.

1.13 "Drug Target" shall mean a gene or other biological target described in the Research Plan or mutually agreed upon by both parties as having potential application for the identification and development of Drug Candidates for the prevention or treatment of SMA.

1.14 "FDA" shall mean the United States Food and Drug Administration (or its successor agency).

1.15 "Field" shall mean the treatment or prevention of [**].

1.16 "First Commercial Sale" shall mean the date of the first commercial sale in a country or region by or on behalf of Company or its Affiliate or Licensee of a Product to another party after Regulatory Approval has been obtained for such Product in such country or region.

1.17 "Hit" shall have the meaning provided in Section 2.4(a).

1.18 "IND" shall mean an Investigational New Drug Application filed with the FDA.

1.19 "Information" shall mean all tangible and intangible techniques, technology, practices, trade secrets, inventions (whether or not patentable), methods, knowledge, know-how, skill, experience, test data and results (including pharmacological, toxicological and clinical test data and results), analytical and quality control data, results or descriptions, software, algorithms, compositions of matter, cells, cell lines, assays, animal models and physical, biological or chemical material.

1.20 "IP Filing Period" shall have the meaning provided in Section 5.4.

1.21 "Lead Candidate" shall have the meaning provided in Section 2.4(a).

1.22 "Lead Optimization" shall mean shall mean a program of medicinal chemistry the intent of which is to develop a Lead Candidate into a compound or formulation suitable as the subject of an IND submission to the FDA.

1.23 "Licensee" shall mean a Third Party to whom Company or any of its Affiliates has granted a license or sublicense of the right to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidate or Product, beyond the mere right to purchase Drug Candidate or Product from Company or its Affiliates.

1.24 "Net Sales" shall mean the gross amounts received by Company and its Affiliates (but not their respective Licensees) following the First Commercial Sale of a Product for sales of such Product to Third Parties that are not Affiliates or Licensees of the selling party (unless such Affiliate or Licensee is the end user of such Product, in which case the amount billed therefor shall be deemed to be the amount that would be billed to a Third Party end user in an arm's-length transaction), less the following items, as allocable to such Product (if not previously deducted from the amount invoiced): (i) bad debts actually written off which are attributable to sales of Products; (ii) trade discounts, credits or allowances; (iii) credits, refunds or allowances additionally granted upon returns, rejections or recalls; (iv) freight, shipping and insurance charges; (v) taxes, duties or other governmental tariffs (other than income taxes); (vi) any payment in respect of sales to any governmental authority in respect of any government-subsidized program, including, without limitation, Medicare and Medicaid rebates; and (vii) distribution, packing, handling and transportation charges for Products to the extent that they are included in the price or otherwise paid by the customer.

1.25 "Patents" shall mean (a) United States and foreign patents, re-examinations, reissues, renewals, extensions and term restorations, and foreign counterparts thereof, and (b) pending applications for United States and foreign patents, including, without limitation, provisional applications, continuations, continuations-in-part, divisional and substitute applications, including, without limitation, inventors' certificates, and foreign counterparts thereof.

1.26 "Principal Scientist" shall mean Dr. Stuart Peltz.

1.27 "Product" shall mean a pharmaceutical product comprising or containing a Drug Candidate, including, in each case, all formulations, line extensions and modes of administration thereof.

1.28 "Product Revenues" shall mean Net Sales of Products by Company and its Affiliates, plus all royalties, license fees, milestone payments, annual maintenance fee or similar payment or consideration paid by a Licensee to Company or its Affiliates in consideration for the grant by Company or its Affiliate of a license to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidates or Products (with any of the foregoing consideration received by Company other than in the form of cash to be valued at its fair market value as of the date of receipt), minus any payments attributable to Product that are made by Company or its Affiliates in respect of a Third Party Patent License; *provided, however,* that "Product Revenues" shall in any event exclude any funds paid to directly support research and/or development actually being performed by Company or its Affiliates (in amounts that are commercially reasonable in light of the research and/or development services being performed), and payments for equity or debt

securities of Company or its Affiliates (except to the extent such payments exceed the fair market value of such securities upon date of receipt, in which event such excess over fair market value shall be included in the calculation of Product Revenues).

1.29 "Regulatory Approval" shall mean any and all approvals (including pricing and reimbursement approvals), licenses, registrations or authorizations of any kind by the FDA or other applicable regulatory authority outside the U.S. necessary for the development, pre-clinical

and/or human clinical testing, manufacture, quality testing, supply, use, storage, importation, export, transport, pricing, marketing and/or sale of a Product for use in the Field.

1.30 "Research" shall mean the activities conducted pursuant to the Research Plan.

1.31 "Research Funds" shall mean all amounts actually paid to Company pursuant to Section 4.1.

1.32 "Research Invention" shall mean any new and useful composition of matter, process, product by process, machine or manufacture, know-how, discovery, improvement, or other intellectual property or any new and useful improvement thereof, whether or not patentable, discovered, conceived, developed or first reduced to practice in the conduct of the Research.

1.33 "Research Milestone" shall have the meaning provided in Section 2.2.

1.34 "Research Plan" shall mean the research plan attached hereto as ***Exhibit A***, which is incorporated herein by this reference, as such research plan may be modified from time to time by mutual written agreement of the Foundation and Company.

1.35 "Research Project" shall mean any one of the constituent research projects that make up the Research, each identified by sequential lettering in the Research Plan.

1.36 "Research Term" shall have the meaning provided in Section 2.6.

1.37 "Research Tool" shall mean a Research Invention that may contribute to the identification or development of products useful in the Field, and that is none of the following: (a) Drug Candidate(s) identified by Company (or any of its corporate partners, Licensees, or sublicensees); (b) Product(s) based on or containing such Drug Candidate(s); or (c) Company Base IP. For the avoidance of doubt, the parties do not intend the definition of Research Tool to apply, in whole or in part, to any aspect of PTC's GEMS technology.

1.38 "Reversionary License" shall have the meaning provided in Section 6.1(c).

1.39 "SMA" shall mean spinal muscular atrophy.

1.40 "SMA Research Tools" shall mean any research tools of the Foundation or its Affiliates, or any Third Parties with which SMA has a relationship, which might be necessary or useful for the Research.

1.41 "Third Party" shall mean any entity other than the Foundation or Company or an Affiliate of the Foundation or Company.

1.42 "Third Party Patent License" shall have the meaning provided in Section 3.2.

2. CONDUCT OF THE RESEARCH.

2.1 Objective. Subject to the terms and conditions of this Agreement, the parties agree that, during the Research Term, Company shall perform the Research in accordance with the Research Plan, and each party shall contribute the materials and services specified therein, with the goal of identifying and developing small molecule therapeutics for use in the Field.

2.2 Research Plan; Contributions. The Research Plan sets forth the activities proposed to be conducted by Company, together with an anticipated schedule for completion of such activities. Company agrees to use commercially reasonable efforts to achieve the research milestones (the "***Research Milestones***") and research goal(s) described in Exhibit B (attached hereto) on the schedule set forth therein and to incorporate feedback from the Foundation's scientific advisors. The parties will jointly review the research goals, activities and schedule set forth in the Research Plan and may, by mutual written agreement, amend the Research Plan from time to time during the course of the Research Term and, in connection therewith, may (i) modify the funding amounts and schedule set forth in Section 4.1, (ii) add additional Research Milestones or goals to Exhibit B, or (iii) provide for Additional Payments, as appropriate. Each party shall contribute to the Research the materials and services specified in the Research Plan, and the Foundation shall use commercially reasonable efforts to assist Company in obtaining favorable licensing terms to SMA Research Tools necessary or useful for the Research.

2.3 Principal Scientist. The Principal Scientist is considered essential to the Research being performed, and no substitution may be made without the prior written agreement of the Foundation. If for any reason the Principal Scientist ceases to be employed by Company or otherwise becomes unavailable, or cannot continue to oversee the conduct or completion of the Research, Company will propose a successor whose appointment as Principal Scientist shall be subject to the approval of the Foundation, such approval not to be unreasonably withheld. If the parties are unable to agree upon a successor within 90 days after the Principal Scientist ceases his involvement in the Research, this Agreement may be terminated by the Foundation pursuant to Section 7.3.

2.4 The Research.

(a) During the Research Term, Company shall conduct each of the Research Projects in accordance with this Agreement and the Research Plan. Company shall disclose the results of all Research activities to the Foundation in accordance with Section 2.7. Company may select, after disclosing the applicable criteria to the Foundation, one or more compounds that have been validated in secondary assay(s) and have suitable *in vitro* potency, or otherwise meet the criteria set forth in the Research Plan (or otherwise mutually agreed upon by the parties) for further evaluation (each such compound being hereinafter referred

to as a ***"Hit"***), following which, as more fully described in the Research Plan, Company shall: (i) assess each Hit (and, as the parties deem appropriate consistent with the Research Plan, any analog, derivative or formulation thereof) with the goal of identifying one or more compounds that have suitable properties for administration to humans (each such compound being hereinafter referred to as a ***"Lead Candidate"***); and (ii) evaluate and, if appropriate based on such evaluation, optimize each Lead Candidate for therapeutic administration to humans.

(b) The parties shall mutually agree upon a strategy for medicinal chemistry follow-up on Lead Candidates and further pharmacology studies, formulation development, safety and toxicity studies, dosing studies or other preclinical work at Company, or at Company's option, through external collaboration or licensing with a Third Party. As promptly as practicable after identification of one or more Lead Candidates, Company shall provide the Foundation with total cost estimates for continued Lead Optimization and development of such Lead Candidates, and the Foundation may elect to fund, in the form of cash payments to Company, some, all or none of this work upon reasonable advance notice to Company. In addition, the Foundation may act to secure funding from Third Parties, and/or assist Company to obtain alternative sources of external funding, and in each case such funding would be administered through and governed by this Agreement as specified in a written agreement with any such Third Party, such Agreement to specify the impact of such alternative sources of funding on the payment obligations of the Company under Section 4.3.

(c) Company shall disclose the results of all Research activities regarding Hits and Lead Candidates to the Foundation in accordance with Section 2.7, and the parties shall consult with each other with the objective of identifying at least one Drug Candidate suitable for progression to the preparation and filing of an IND in the Field and, contingent on the effectiveness of such IND, progressing such Drug Candidate into human clinical trials in the most expeditious manner.

2.5 Performance Standards. Company shall conduct the Research in good scientific manner, and in compliance in all material respects with the requirements of applicable laws and regulations and with applicable good laboratory practices, to attempt to achieve its objectives efficiently and expeditiously. Company shall maintain (either as its own internal resources, or via subcontract) laboratories, offices and all other facilities reasonably necessary to carry out the activities to be performed by it pursuant to the Research Plan. In conformity with standard pharmaceutical and biotechnology industry practices and the terms and conditions of this Agreement, Company shall prepare and maintain, or shall cause to be prepared and maintained, complete and accurate written records, accounts, notes, reports and data with respect to activities conducted pursuant to the Research Plan. Upon reasonable advance notice, Company agrees to make its employees and non-employee consultants reasonably available at their respective places of employment to consult with the Foundation on issues or questions arising during the Research Term.

2.6 Research Term. The initial phase of the Research is expected to require one year to reach the primary overall objective of Lead Candidate identification. The parties may, from time to time, on a Research Project-by-Research Project basis, extend or modify the Research Term by mutual written agreement (the initial one-year period and any extensions or modifications together shall hereinafter referred to as the ***"Research Term"***).

2.7 Communication; Research Reports. On a regular basis during the Research Term (but no less frequently than [**]), the parties shall conduct meetings, either in person or by telephone or video conference, to discuss the progress of the Research and strategies for achieving the objectives of the Research in an expeditious manner. Company shall keep the Foundation fully informed as to all results and discoveries (including, without limitation, assay development and all Hits and potential Lead Candidates and Drug Candidates) made in the

course of performing activities under the Research Program at these meetings. In furtherance of the foregoing, on a [**] basis, Company shall prepare, and deliver to the Foundation no later than [**] days after the conclusion of [**] during the Research Term, a reasonably detailed written summary report of the results and progress of the Research during [**] (each, a ***"Research Report"***). In addition, the Foundation may, at its option, during the Research Term, schedule up to [**] formal program review meetings with Company personnel and those of Foundation's Third Party advisors who (i) have agreed to confidentiality restrictions substantially similar to those contained in this Agreement, and (ii) are reasonably acceptable to Company. Such meetings will be held at the times and locations mutually agreed upon by the parties. The purpose of such meetings will be to review the progress of the Research relative to the Research Plan.

2.8 Subcontracts. Company may perform some of its obligations under the Research Plan through one or more subcontractors, provided that (a) none of the rights of either party hereunder are diminished or otherwise adversely affected as a result of such subcontracting, and (b) Company will at all times be responsible for the performance and, except as otherwise agreed by the parties in writing, payment of such subcontractor; provided, however, that the Company may use payments received by it pursuant to Section 4.1 to pay for such subcontractor(s). In determining whether any Company obligations under the Research Plan will be performed in-house or by a Third Party subcontractor, Company shall take into consideration Company's then-current capabilities and the relative efficiency of utilizing such internal capabilities versus Third Party services.

2.9 Additional Screening. The Foundation may request that Company test up to [**] compounds identified by other Foundation partners ***("Third Party Compounds"***) on a blinded basis. Company agrees to test such Third Party Compounds on behalf of the Foundation and to disclose the results of such screening to the Foundation, provided that the relevant assay is already being run by the Company on its own Compounds. Such testing shall be performed pursuant to a separate materials transfer agreement to be negotiated in good faith by the parties prior to provision of any compounds or related information, which agreement shall contain reasonable and customary terms to protect the parties' respective intellectual property rights. Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any Third Party Compound become the property of the Company, nor shall any Third Party Compound become subject to royalty or other reach-through payment obligations to Company or its affiliates as a result of such screening by Company.

3. DEVELOPMENT OF PRODUCTS.

3.1 Clinical Development Strategy. As soon as Company reasonably believes that it has identified a Drug Candidate for which it proposes to file an IND in the Field, Company will notify the Foundation in writing, and the parties will promptly discuss in good faith how to proceed with the clinical development of such Drug Candidate, taking into consideration the interests of SMA patients, the intellectual property and regulatory landscape and the commercial potential of the Drug Candidate. The parties agree to consider in good faith collaborating with the NIH in preclinical or clinical development activities regarding such Drug Candidate. Should Company elect to proceed with clinical development of the Drug Candidate, it may do so directly. In the alternative, at its discretion, the Company may decide to enter into a

collaboration with one or more Third Parties for clinical development and/or commercialization of the Drug Candidate through licensing or other arrangement; *provided, however,* that if the Foundation has funded (or caused to be funded) [**], then until [**], any such collaborations shall be subject to the Foundation's approval (which shall not be unreasonably withheld). If Company wishes to pursue clinical development of a Drug Candidate, the Company will consult with the SMA Foundation on the clinical trial network that will be used. Although the parties currently expect to use the clinical trial network established by the Foundation, the clinical trial network to be used shall be determined in good faith by Company in its reasonable judgment. For any Drug Candidate for which it files an IND, Company agrees to consider in good faith whether to obtain, (a) "Orphan Product" designation from the FDA, and (b) research funding from the FDA's Office of Rare Diseases to support human clinical trials conducted for such Drug Candidate. The parties acknowledge that if the Drug Candidate is [**], or [**] due to [**] and [**], investment by Company in further development of such Drug Candidate may not be in the best interests of Company's stockholders, and therefore shall not be required under this Agreement, and the failure to engage in such further development shall not be the basis of a Reversionary License under Section 6.1(c). In such case, the parties may elect to enter into an additional sponsored research agreement under which the Foundation would provide funding for further development efforts by Company, but neither party shall have any obligation to enter into such additional agreement.

3.2 Conduct of Clinical Development. Except as set forth in Section 3.1 above or as otherwise agreed by the parties in writing, Company shall be responsible for clinical development of any Drug Candidate for which Company files an IND. Company shall use commercially reasonable efforts to develop and commercialize (whether directly, through an Affiliate, or in collaboration with one or more Third Parties, through licensing or some combination of the foregoing) at least one Product. The parties anticipate that an IND will be submitted within [**] years of commencement of IND-enabling toxicology studies for a Lead Candidate, but the parties acknowledge that [**], and therefore [**], to be a [**]. Notwithstanding the preceding provisions of this Section 3.2, in no event shall Company have any obligation (i) to pursue clinical development or commercialization of any Drug Candidate which is [**], or which [**] the [**] due to its [**] and [**], or (ii) in the absence of complete funding by (or arranged by) the Foundation, to pursue clinical development or commercialization of any Drug Candidate which is not, [**]. In addition, Company shall not be obligated to pursue clinical development or commercialization of a Drug Candidate if the pharmaceutical preparation, composition of matter, method of manufacture and/or method of use of such Drug Candidate is covered by Patents of a Third Party, unless a license under such Third Party Patents is available to Company (or its Affiliate or Licensee, as applicable) on commercially reasonable terms (a ***"Third Party Patent License"***).

3.3 Disclosure Regarding Company Efforts. Company will keep the Foundation appropriately informed about clinical trial progress and commercialization efforts with respect to Products, and in any event, Company shall provide the Foundation with [**] written reports summarizing any significant development or commercialization events that have occurred during the applicable [**]-month period, provided that such reports may be incorporated into any Research Reports then being prepared and delivered under Section 2.7.

4. PAYMENTS.

4.1 Research Funding by the Foundation. For the conduct of the Research, and subject to the completion of the applicable Research Milestones described in Exhibit B (attached hereto), the Foundation shall pay a total of US$[**] to Company on the schedule specified below:

(a) within [**] days after the Effective Date, the Foundation will pay to Company US$[**];

(b) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 1 in Exhibit B attached hereto, the Foundation will pay Company US$[**];

(c) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 2 in Exhibit B attached hereto, the Foundation will pay Company US$[**]; and

(d) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 3 in Exhibit B attached hereto, the Foundation will pay Company US$[**].

The Foundation may delay any payment until such time as the milestones in the Research Plan are met (or as may otherwise be mutually agreed in writing). For purposes of clarification, the foregoing payments shall be non-refundable, and each of the foregoing payments shall be payable only once. The Foundation acknowledges that the foregoing payments represent only a portion of the total cost of performing the Research. Notwithstanding the foregoing,, except as agreed pursuant to Section 4.2, the Foundation will not be obligated to pay any additional amounts in connection with the Research.

4.2 Additional Payments. In addition to the amounts specified in Section 4.1, upon mutual written agreement of the parties, the Foundation may make, or cause to be made, additional research funding payments to Company in connection with any modification of the Research Plan.

4.3 Milestone Donation by Company. Within [**] days after the end of the first fiscal quarter in which Company has received an aggregate of US$[**] in Product Revenues, Company shall make a payment to the Foundation (or, at the Foundation's option, one or more other non-profit organizations or academic or research institutions designated by the Foundation in writing) in the applicable amount set forth below pursuant to clause (a), (b) or (c), whichever **one** (and only one) of the following applies:

(a) [**];

(b) [**]; or

(c) [**].

In addition to the foregoing milestone payments, and provided that the Foundation provided funding for Lead Optimization of Products hereunder at the level set forth in the first paragraph of 4.3(c), within [**] days after the end of the first calendar year during which Company has received an annual aggregate in that year of US$[**] in Product Revenues, Company shall make a payment to the Foundation equal to 100% of the sum of the Research Funds and the Additional Payments. For the avoidance of doubt, such additional payment shall be a one-time payment only, regardless of any additional Product Revenues.

If [**] in good faith believes that making the applicable payment(s) specified in this Section 4.3 on the schedule set forth above will prevent Company from achieving a reasonable profit margin on commercial sales of Products, [**] may reduce any such payments due in the applicable calendar or fiscal quarter by [**]%, or such other reduction as the parties shall in good faith agree, with any reduction carried forward on a quarter-by-quarter basis (subject to the same reductions in each subsequent quarter) until paid in full.

4.4 Reporting of Product Revenues. From and after such time as Company first receives any Product Revenues and until such time as Company has paid in full the amount due under Section 4.3 (if any), Company shall deliver to the Foundation (or a Third Party designated in writing by the Foundation) quarterly written reports of Product Revenues received by Company and its Affiliates, which reports shall indicate the total Product Revenues received. Company shall keep, and shall cause its Affiliates to keep, complete and accurate records pertaining to the receipt of Product Revenues in sufficient detail to permit the Foundation to confirm the accuracy of such reports.

4.5 Exchange Rate; Manner and Place of Payment. All payments hereunder shall be payable in U.S. dollars. When conversion of payments from any foreign currency is required for purposes of calculating Product Revenues, such conversion shall be at the exchange rate used by Company (or, where applicable, a Licensee) throughout its accounting system (which shall, in any event, be commercially reasonable) during the quarter for which such report is due. All payments owed under this Agreement shall be made by check, or by wire transfer in immediately available funds to a bank and account designated in writing by the party entitled to receive payment, unless otherwise specified in writing by such party.

4.6 Taxes. Each party will pay any and all taxes levied on account of any payments made to it under this Agreement out of the amounts it is to receive hereunder. If any taxes are required to be withheld by the party making payment, such party will (a) deduct such taxes from the payment made by it, (b) timely pay the taxes to the proper taxing authority, (c) send proof of payment to the other party and certify its receipt by the taxing authority within [**] days following such payment, and (d) be deemed to have paid such amount to the other party hereunder.

4.7 Audits. The Foundation shall have the right to cause an independent, certified public accountant reasonably acceptable to Company to audit the records of Company and its Affiliates to confirm the accuracy of Company's reports of Product Revenues for a period covering not more than the preceding [**] years. Such audits may be exercised during normal business hours upon reasonable prior written notice to Company and no more than [**] per year. Prompt adjustments shall be made by the parties to reflect the results of such audit. The

Foundation shall bear the full cost of such audit unless such audit discloses an underreporting of Product Revenues by Company of more than [**]% during any calendar year, in which case, Company shall bear the full cost of such audit.

5. CONFIDENTIALITY.

5.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the parties, the parties agree that, during the Research Term and for a period of [**] years thereafter, each party (the ***"Receiving Party"***) will maintain in confidence all Confidential Information disclosed to it by the other party (the ***"Disclosing Party"***), provided that, with regard to Confidential Information which is trade secret information, such obligation shall extend thereafter until such information is no longer a trade secret of the Disclosing Party. The Receiving Party may use the Confidential Information of the Disclosing Party only to the extent required to accomplish the purposes of this Agreement. The Receiving Party shall use at least the same standard of care as it uses to protect proprietary or confidential information of its own to ensure that its employees, agents, consultants and other representatives do not disclose or make any unauthorized use of the Disclosing Party's Confidential Information. Each party will promptly notify the other upon discovery of any unauthorized use or disclosure of the other party's Confidential Information.

5.2 Exceptions. The obligations of confidentiality contained in Section 5.1 will not apply to the extent that it can be established by the Receiving Party by competent proof that such Confidential Information: (a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement; (d) is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or (e) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

5.3 Authorized Disclosure. Notwithstanding any other provision of this Agreement, disclosure of Confidential Information shall not be precluded if such disclosure is in response to a valid order of a court or other governmental body of competent jurisdiction of the United States or any political subdivision thereof or is otherwise required by law or regulation; *provided, however,* that the Receiving Party shall, to the extent practicable, first have given notice to the Disclosing Party and shall have made a reasonable effort to obtain a protective order requiring that the Confidential Information so disclosed be used only for the purposes for which the order was issued or the law or regulation required or to seek other confidential treatment of such information.

5.4 Publication. The parties acknowledge and agree that the SMA research community and medical professionals treating SMA patients will benefit from disclosure of the Data as soon as practicable. Accordingly, should the Foundation wish to publish any Confidential Information contained in a Research Report, it shall provide Company with [**] days' advance notice of such publication (the ***"IP Filing Period"***) to allow Company to file

patent applications covering the Company Technology disclosed in such Research Report; *provided, however,* that at Company's reasonable request, the IP Filing Period shall be extended for an additional [**] days if necessary for the filing of appropriate patent applications covering Company Technology disclosed in or apparent from such Research Report. During the IP Filing Period, the Foundation shall maintain as confidential the Data and the Research Report provided to Foundation by Company. Notwithstanding the foregoing, in no event shall Foundation disclose the structures of any chemical compound being researched or developed by Company in any publication or other public forum without the prior written consent of Company. Except as expressly set forth in this Agreement, the Foundation shall not have the right to use the Data to develop, commercialize, market or sublicense any commercial offering of any product or service based on the Data. The Company shall provide in each [**] Research Report a summary section which is suitable for immediate public disclosure and the Foundation may release copies of such portions of each Research Report and supporting Data to any Third Party investigator who requests such material from the Foundation in writing; *provided, however,* that said Third Party investigator first executes Company's non-disclosure agreement (it being understood that such non-disclosure agreement will not prohibit said Third Party investigator from applying his or her knowledge of the Data to further SMA research and/or to treatment of SMA

patients, but will prohibit him or her from transferring such Data except as incidental and necessary to treating SMA patients). The Foundation will treat all other Data in each Research Report as Company Confidential information. To the extent that any journal or other forum in which the Foundation proposes to publish or disseminate the Data requires the authorship or participation of one or more Company employees or contractors who participated in the Research or in the development of a Drug Candidate or Product, Company shall use commercially reasonable efforts to cause such individuals to cooperate with the Foundation in making such publication and, as necessary or appropriate, to be named as authors (or co-authors) of such publication. Any publication or presentation of Data in any Research Report shall acknowledge each party's contribution thereto in accordance with customary scientific practice.

5.5 Publicity; Regulatory Disclosures. It is understood that the parties intend to issue a joint press release announcing the execution of this Agreement, and the parties agree that each party may desire or be required to issue subsequent press releases or make disclosures in regulatory filings relating to this Agreement or activities hereunder. The parties agree to consult with each other reasonably and in good faith with respect to the text and timing of such press releases or other disclosures prior to the issuance thereof, provided that a party may not unreasonably withhold consent to such releases or disclosures, and that either party may issue such press releases or disclosures as it determines, based on advice of counsel, are reasonably necessary to comply with laws or regulations or for appropriate market disclosure. In addition, following the initial joint press release announcing this Agreement, either party shall be free to disclose, without the other party's prior written consent, the existence of this Agreement, the identity of the other party and those terms of the Agreement which have already been publicly disclosed in accordance with this Section 5.5.

6. OWNERSHIP AND USE OF DATA AND INTELLECTUAL PROPERTY.

6.1 Ownership; Reversionary License.

(a) Data. Company shall solely own all data generated as a result of the Research (the ***"Data"***).

(b) Company Technology. Company shall solely own all Company Technology.

(c) Reversionary Licenses to Data and Company Technology. With respect to Research Projects in which [**], in the event that:

(i) Company elects not to continue the Research or subsequent development of at least one Drug Candidate or Product relating to any Research Project in the Field; or

(ii) Company fails to use commercially reasonable efforts to conduct development and commercialization of at least one commercially viable Drug Candidate arising in the Field, and is unable to remedy such failure to comply within [**] days after notice thereof from the Foundation; or

(iii) Company is otherwise in material breach of this Agreement with respect to such Research Project and is unable to remedy such breach within [**] days after notice of such breach from the Foundation;

then, in any such case, Company shall, and it hereby does, grant to the Foundation an exclusive worldwide license, including the right to grant sublicenses, under any Company Technology resulting from such Research Project that relates to a pharmaceutical preparation, composition of matter, method of manufacture and/or method of use of such Drug Candidate Lead Candidates, Drug Candidates and Products in the Field, solely for the purpose of researching, developing, making, having made, using, selling, having sold, offering for sale and importing Drug Targets, Lead Candidates, Drug Candidates and Products in the Field (each such license with respect to a particular Research Project being referred to herein as a ***"Reversionary License"***), and use of such Data by the Foundation or its sublicensee(s) as reasonably necessary or appropriate to exploit such Reversionary License shall not represent a violation of Section 5.1 above; *provided*, *however,* that in the case of Research Project B such license shall not be granted if (x) the Company project team, with the concurrence of the Foundation or its advisors, determines that the compounds identified in the conduct of Research Project B are not more active than the [**] in the applicable assay(s), or are more active but [**] for [**], and Company does not pursue development and commercialization of such compounds; or (y) the Foundation chooses not to [**] of Research Project B [**]; and *provided, further,* that the Reversionary License with respect to a particular Research Project shall not become effective (I) if the parties mutually agree, after good faith discussions based on [**] of such Research Project, that such Research Project [**], (II) Company [**] in such Research Project that [**], or (III) Company [**] in such Research Project that [**] but the [**] of the research for such Research Project. If the Reversionary License covers a Product which, as of the date of effectiveness of the Reversionary

License, has [**], and the Reversionary License was granted pursuant to Section 6.1(a)(i), then Foundation [**] a [**] of such Product in the [**] such Product [**] in [**]. In any [**] in the [**], the Reversionary License [**].

(d) Research Tools. The parties acknowledge that the SMA research and clinical communities will benefit from the availability of Research Tools. Company agrees to use commercially reasonable efforts to make Research Tools Controlled by it available to members of the spinal muscular atrophy research and clinical communities (excluding for-profit entities engaged in pharmaceutical research and development) for research or educational purposes on commercially reasonable terms as promptly as practicable following request by the Foundation or such person (it being understood that neither Company nor its corporate partners shall charge reach-through royalties with respect to drugs discovered by such persons using Research Tools, so long as such drugs themselves are not covered by Company Technology); *provided*, *however,* that Company shall not have any obligation to provide such access before the publication of patent applications containing claims (adequately supported by written description) that cover the relevant Research Tool. Notwithstanding the foregoing, if Company believes in good faith that [**], then Company shall so notify the Foundation in writing, and the parties shall discuss in good faith how to proceed.

6.2 Patent Filings. (a) Company shall file, prosecute and maintain all Patents on the Company Technology at its sole expense. Notwithstanding the foregoing, if Company is obligated to make the Reversionary License to the Foundation as described in Section 6.1(c) above, then the Foundation shall have the right, itself or through its designee, to file, prosecute and maintain Patents licensed under the Reversionary License at its sole expense; *provided*, *however,* that if [**], and [**], and further provided, that the Company shall have reasonable rights of comment and consultation on all such prosecution and maintenance activities, (b) Each of Company and Foundation shall execute all papers and instruments, and require its employees and contractors to execute all papers and instruments, so as to enable the other party to exercise the rights set forth in Section 6.2(a).

6.3 SMA Research Tools. Foundation shall use commercially reasonable efforts to assist Company in obtaining favorable licensing terms for access to SMA Research Tools necessary or useful for the conduct of the Research.

6.4 No Other License. Other than any license granted pursuant to Section 6.1(c), no license is granted or implied with respect to any Company Technology or Data for any use.

7. TERM; TERMINATION.

7.1 Term. The term of this Agreement shall commence on the Effective Date and shall continue until expiration of the Research Term, unless this Agreement is earlier terminated in accordance with this Article 7.

7.2 Termination for Cause. Each party shall have the right to terminate this Agreement upon 60 days' prior written notice to the other upon the occurrence of any of the following:

(a) Upon or after the bankruptcy, insolvency, dissolution or winding up of the other party (other than a dissolution or winding up for the purpose of reorganization); or

(b) Upon or after the breach of any material provision of this Agreement by the other party if the breaching party has not cured such breach within the 60-day period following written notice of termination by the non-breaching party.

7.3 Termination Upon Principal Scientist's Unavailability. The Foundation may terminate this Agreement upon 30 days' prior written notice to Company in the event the Foundation and Company are unable to agree upon a suitable replacement for the Principal Scientist pursuant to Section 2.3; *provided, however,* that termination in accordance with this Section 7.3 will not trigger the grant of any Reversionary License under Section 6.1. In the event of a termination of this Agreement pursuant to this Section 7.3, and notwithstanding any other provision of this Agreement to the contrary (including but not limited to Section 7.4), only the provisions of Sections 6.1(a), 6.1(b), 6.2(b), this Section 7.3, the first sentence of Section 6.2(a), and Articles 1, 5, 8, and 9 will survive such termination.

7.4 Consequences of Expiration or Termination. Expiration or termination of this Agreement will not relieve the parties of any obligation accruing prior to such expiration or termination (including, without limitation, any accrued obligation of the Foundation to make payments pursuant to Section(s) 4.1 and/or 4.2). Except as otherwise provided in Section 7.3, and notwithstanding any other provision of this Agreement to the contrary, the provisions of Sections 4.3, 4.4, 4.5, 4.6, 4.7, 7.4, and 7.5, and Articles 1, 5, 6 (to the extent applicable), 8 and 9 will survive expiration or termination of this Agreement.

7.5 Rights in Bankruptcy. All rights and licenses granted under or pursuant to this Agreement by Company are, and will otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code, licenses of right to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The parties agree that the Foundation, to the extent it receives a Reversionary License pursuant to Section 6.1(c), as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code. The parties further agree that, in the event of the commencement of a bankruptcy proceeding-by or against Company under the U.S. Bankruptcy Code, the Foundation will be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, and same, if not already in the Foundation's possession, will be promptly delivered to the Foundation (a) upon any such commencement of a bankruptcy proceeding upon its written request therefor, unless Company elects to continue to perform all of its obligations under this Agreement, or (b) if not delivered under clause (a) above, following the rejection of this Agreement by or on behalf of Company upon written request therefor by the Foundation.

8. INDEMNIFICATION.

8.1 Indemnification by Company. Company hereby agrees to save, defend, indemnify and hold harmless the Foundation, its trustees, officers, employees and agents (each, a ***"Foundation Indemnitee"***) from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expenses and attorneys' fees ***("Losses")***, to which a Foundation Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Losses arise directly or indirectly out of (a) the development, manufacture, handling, storage, sale or other disposition of any Drug Candidate or Product by Company, its Affiliate(s) or Licensee(s), or (b) the breach of this Agreement by Company or the gross negligence or willful misconduct of Company, except in each case to the extent such Losses result from (x) the breach of this Agreement by the Foundation or the gross negligence or willful misconduct of any Foundation Indemnitee, or (y) the activities of the Foundation or its agents or employees in connection with any Research Project or related Drug Candidate or Product after the Foundation has received a Reversionary License in connection with such Research Project under Section 6.1(c) ("Reversionary License Activities").

8.2 Conditions to Indemnification. The obligations of Company under Section 8.1 are conditioned upon the Foundation's delivery of written notice to Company of any potential Losses promptly after the Foundation becomes aware of such potential Losses. Company shall have the right to assume the defense of any suit or claim related to the Losses if it has assumed responsibility for the suit or claim in writing. If Company defends the suit or claim, the Foundation may participate in (but not control) the defense thereof at its sole cost and expense.

8.3 Settlements. Neither party may settle a claim or action related to any Losses subject to indemnification under Section 8.1 without the consent of the other party, if such settlement would impose any monetary obligation on the other party or require the other party to submit to an injunction or otherwise limit the other party, its Affiliates, trustees, employees, agents, officers or directors.

8.4 Insurance. During any period when Company, its Affiliate or any Licensee is clinically developing or commercializing any Drug Candidate or Product and for [**] years thereafter, Company, at its own expense, shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall name the Foundation as an additional insured with respect to such insurance, with respect to losses arising out of or related to the activities contemplated under this Agreement. Company shall provide a certificate of insurance evidencing such coverage to the Foundation upon request.

8.5 Liability of the Foundation. The Foundation assumes any and all risk of personal injury and property damage attributable to the practice by the Foundation, its trustees, officers, employees or agents, or its designee or sublicensee of any license granted by Company to the Foundation hereunder, the breach of this Agreement by the Foundation or any Foundation Indemnitee, or the gross negligence or willful misconduct of any Foundation Indemnitee. Furthermore, the Foundation assumes any and all risk of Losses (as defined above) in connection with any Reversionary License Activities.

9. MISCELLANEOUS.

9.1 Assignment. Except as expressly provided hereunder, neither this Agreement nor any rights or obligations hereunder may be assigned or otherwise transferred by either party without the prior written consent of the other party (which consent shall not be unreasonably withheld). Notwithstanding the foregoing, the Foundation shall have the right to assign or transfer any or all of its rights or obligations under this Agreement to a Third Party that is a non-profit organization upon written notice to Company, provided that the Foundation shall remain liable for any payment obligations accruing hereunder to the extent that such Third Party does not comply with such obligations. Company shall have the right to assign or transfer any or all of its rights or obligations under this Agreement to a Third Party in connection with the transfer or sale of all or substantially all of the portion of Company's business to which this Agreement relates, or in the event of Company's merger or consolidation or change in control or similar transaction or the creation of a special purpose corporation or research and development limited partnership or a joint venture. The rights and obligations of the parties under this Agreement shall be binding upon and inure to the benefit of the successors and permitted assigns of the parties. Any assignment not in accordance with this Agreement shall be void.

9.2 Force Majeure. Neither party shall be held liable or responsible to the other party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of the Agreement when such failure or delay is caused by or results from causes beyond the reasonable control of the affected party, including, without limitation, fire, floods, earthquakes, natural disasters, embargoes, war, acts of war (whether war be declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, acts of God or acts, omissions or delays in acting by any governmental authority or the other party.

9.3 Governing Law. This Agreement shall be governed by, and construed and enforced in accordance with, the laws of the State of New York, without regard to its choice of law provisions; provided, however, that with respect to intellectual property filings, such filings will be governed by the federal laws of the United States, or, if outside the United States, by the applicable intellectual property laws of the relevant jurisdiction(s).

9.4 Waiver. Except as specifically provided for herein, the waiver from time to time by either party of any right or failure to exercise any remedy shall not operate or be construed as a continuing waiver of the same right or remedy or of any other of such party's rights or remedies provided under this Agreement.

9.5 Severability. In case any provision of this Agreement shall be invalid, illegal or unenforceable, the validity, legality and enforceability of the remaining provisions shall not in any way be affected or impaired thereby.

9.6 Independent Contractors. It is expressly agreed that Company and the Foundation shall be independent contractors and that the relationship between the two parties shall not constitute a partnership, joint venture or agency of any kind. Neither party shall have the authority to make any statements, representations or commitments of any kind, or to take any

action, which shall be binding on the other party, without the prior written consent of the other party.

9.7 Notices. All notices and other communications provided for hereunder shall be in writing and shall be mailed by first-class, registered or certified mail, postage paid, or delivered personally, by overnight delivery service or by facsimile, with confirmation of receipt, addressed as follows:

If to the Foundation:
Spinal Muscular Atrophy Foundation
1776 Broadway, 22nd Floor
New York, NY 10019
Fax: (212) 247-3079
Attention: Ms. Cynthia Joyce, Executive Director

With a copy to:
Cooley Godward LLP
4401 Eastgate Mall
San Diego, CA 92121
Fax: (858) 550-6420
Attention: Jane K. Adams, Esq.

If to Company:
PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080-2449
Fax: 908-222-7231
Attention: Mark Boulding, Senior Vice President and General Counsel

With an email copy to: legal@ptcbio.com

Either party may by like notice specify or change an address to which notices and communications shall thereafter be sent. Notices sent by facsimile shall be effective upon confirmation of receipt, notices sent by mail or overnight delivery service shall be effective upon receipt, and notices given personally shall be effective when delivered.

9.8 Entire Agreement; Amendment. This Agreement (including the Exhibits hereto, as such Exhibits may be amended from time to time by mutual written agreement of the parties) sets forth all of the agreements and understandings between the parties hereto with respect to the subject matter hereof,

and supersedes and terminates all prior agreements and understandings between the parties with respect to the subject matter hereof. There are no other agreements or understandings with respect to the subject matter hereof, either oral or written, between the parties. Except as expressly set forth in this Agreement, no subsequent amendment, modification or addition to this Agreement shall be binding upon the parties hereto unless reduced to writing and signed by the respective authorized officers of the parties.

9.9 Headings; Section References. The captions contained in this Agreement are not a part of this Agreement, but are merely guides or labels to assist in locating and reading the

several Articles and Sections hereof. Section references herein are to the corresponding Sections of this Agreement unless otherwise indicated.

9.10 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

[REMAINDER OF THIS PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the Effective Date.

SPINAL MUSCULAR ATROPHY FOUNDATION	**PTC THERAPEUTICS INC.**
By: /s/ Loren A. Eng	By: /s/ Stuart Peltz
Printed Name: Loren A. Eng	Printed Name: Stuart Peltz
Title: President	Title: President and CEO

[SIGNATURE PAGE TO SPONSORED RESEARCH AGREEMENT]

EXHIBIT A

RESEARCH PLAN

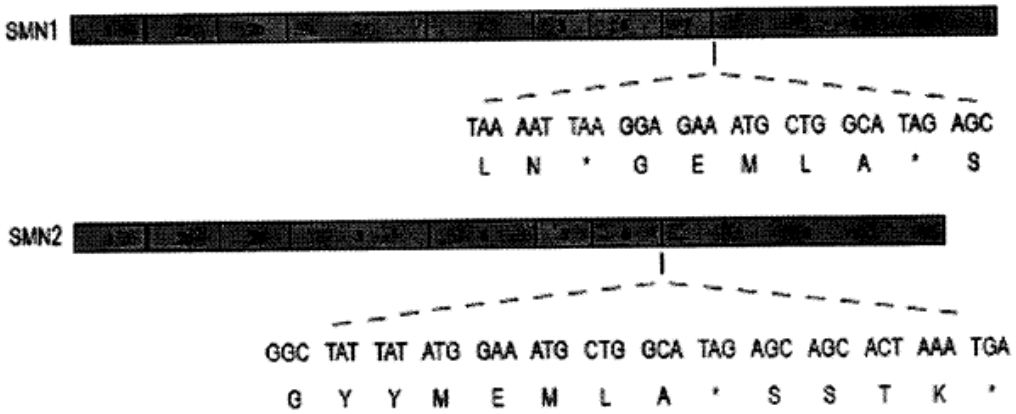
PTC proposes to collaborate with the SMA foundation in order to identify new therapeutics for the treatment of spinal muscular atrophy (SMA). PTC will utilize its expertise, its platform technologies, and compounds to identify new drugs to treat SMA. Three programs for the discovery and development of drugs to treat SMA are contemplated:

[**].

Overview of SMA

Spinal muscular atrophy (SMA) is a common autosomal recessive neurodegenerative disease characterized by degeneration of motor neurons of the anterior horn of the spinal cord resulting in muscle weakness and atrophy. SMA can be subdivided into three clinical groups on the basis of age of onset and severity of the symptoms. The acute type I form is characterized by severe, generalized muscle weakness and hypotonia that is seen either at birth or within the first 3 months of life. Death from respiratory failure usually occurs within the first 2 years. Type II children are able to sit, although they cannot stand or walk unaided. They suffer significant respiratory morbidity and earlier mortality. Type III patients have proximal muscle weakness, starting after age of 2. They generally have a milder course, with the potential for normal life expectancy.

SMA results from reduced expression of survival motor neuron protein (SMN). The SMN gene is duplicated as an inverted repeat on human chromosome 5. The telomeric copy of SMN (SMN1) is deleted or mutated in over 98% of SMA patients. These patients retain at least one copy of the centromeric SMN gene called SMN2. The SMN2 gene has a mutation such that SMN2-derived transcripts are alternatively spliced and encode a truncated protein lacking exon 7 (SMNDExon7). The number of SMN2 copies strongly correlates with severity of SMA.



The quantity of a particular protein synthesized in a given time depends on both the cellular concentration of its mRNA and how efficiently the mRNA is used by cellular

translational apparatus. Multiple cellular mechanisms that affect mRNA availability or utilization are major regulators of protein production and are known as post-transcriptional control processes. PTC targets these processes in its drug discovery efforts. The mRNA sequences that are often found the non-coding regions, known as "untranslated regions" or UTRs. The region before the protein coding region is known as the 5'-UTR, while the region following the protein coding region is known as the 3'-UTR. The largest number of known post-transcriptional control determinants map to the 5'- and 3'-untranslated regions of an mRNA. Post-transcriptional regulation occurs through interaction of cellular factors with sequence elements including secondary structures, protein-binding sites, upstream open reading frames, internal ribosome entry sites, and poly(A) tail.

Examination of the 5' and 3' untranslated regions of SMN2 mRNA reveals a number of sequence elements that strongly suggest that the SMN2 mRNA is post-transcriptionally regulated. The 5' UTR of SMN2 contains a regulatory element known as an upstream open reading frame (uORF). Several lines of evidence strongly suggest that uORFs in the 5' UTRs of mRNAs regulate gene expression by modulating efficiency of translation and mRNA stability. The SMN2 5' UTR also contains 63% of C and G nucleotides suggesting a high degree of secondary structure, which can be an excellent platform for binding of proteins that regulate ribosome scanning and, therefore, translation efficiency. The 3' UTR of SMN2 is 559 nucleotides in length and contains several conserved elements that serve as protein binding sites that are involved in regulating translation efficiency and mRNA stability.

The GEMS technology platform

The GEMS technology platform (Gene Expression Modulation by Small-molecules) targets the post-transcriptional control mechanisms in order to identify small molecules that alter gene expression to increase or decrease protein levels. Compounds identified by the GEMS technology modulate the post-transcriptional control mechanisms of gene expression that act through the 5'- 3'-untranslated regions of mRNA. The GEMS technology can be applied to the SMN2 gene to identify small molecules that increase the expression of the SMN2 gene.

Overview of program [**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of four pages were omitted. [**]

Exhibit B

RESEARCH MILESTONES

Milestone 1: [**]

Milestone 2: [**].

Milestone 3: [**].

Goal: A set of compounds characterized from all three Research Projects.

Final

AMENDMENT No. 1 TO SPONSORED RESEARCH AGREEMENT

This first amendment ("First Amendment") to the Sponsored Research Agreement is entered into as of the 12th day of October, 2007 (the "Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. ("PTC"), with reference to the following facts and circumstances.

WHEREAS Foundation and PTC are parties to that certain Sponsored Research Agreement dated as of June 1, 2006 (the "Agreement");

WHEREAS PTC has achieved all the initial milestones set forth in Exhibit B to the Agreement, and Foundation has made the payments associated with such milestones under the Agreement;

WHEREAS, the Parties desire to extend the Agreement to allow additional funding by Foundation in connection with continued research focused on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this First Amendment, the Parties agree as follows:

1. Definitions. Except as expressly set forth herein, all capitalized terms used herein and not otherwise defined shall be as defined in the Agreement

2. Additional Research. The Parties agree to the modification of the Research Plan attached as Exhibit A-1 to allow for PTC to perform early structure-activity relationship work on Hits identified under each of the three Research Projects (the "Additional Research"). The goal of such research will be the presentation by PTC of a list characterized Lead Candidates for further discussions with Foundation with respect to prioritization and potential funding of Lead Optimization by Foundation. The expected duration of such research is [**] months. The Foundation shall have the exclusive option (the "Option") to continue funding development of all Lead Candidates presented by the company at the end of the Amendment Term (as defined below) through the identification of a Drug Candidate suitable for an IND filing, subject to the following terms and conditions: (a) the Foundation may exercise the Option by providing written notice to PTC of its intent to so fund development within [**]days following the date on which the Final Report (as defined below) is transmitted to Foundation; (b) following

such written notice, the Parties shall negotiate in good faith for a period of no longer than [**] days the budget and terms and conditions of such proposed funding, and (c) if the Parties are unable to reach agreement within such [**] day period, then Foundation's rights under the Option shall expire. Notwithstanding the foregoing, in no case shall the Option preclude PTC from entering into partnering arrangements or other agreements with commercial partners with respect to the Research Projects, so long as PTC is in compliance with the other terms of the Agreement and this Amendment.

PTC shall conduct such Additional Research in accordance with the terms of the Agreement as amended herein, including but not limited to PTC's obligations under Section 2.5 of the Agreement (captioned "Performance Standards"). In connection with such Additional Research,

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the Research Term shall be extended, without interruption, until the date which is eight (8) months following the Amendment Effective Date (the "Amendment Term").

3. Research Reports. In lieu of the Research Reports that would otherwise be due from PTC under Section 2.7 of the Agreement during the Research Term, PTC shall make the following reports: (a) [**] months following the Amendment Effective Date, a summary report showing progress with respect to the Additional Research and identifying any limiting factors or other considerations that may affect completion of the Additional Research (the "Mid-Stage Report"), and (b) within [**] days of completion of the Additional Research, a final report containing the recommendations by PTC for selection of compounds for further research and potential Lead Optimization (the "Final Report"). In addition, PTC will make itself available for Research Team conference calls following its internal research update meetings, which are expected to occur every [**] weeks, for informal discussion of the program.

4. Additional Payments by Foundation. Foundation shall pay PTC a total of [**] US dollars ($[**]) in partial support for the Additional Research as follows: (a) [**] US dollars ($[**]) within [**] days of the Amendment Effective Date; (b) [**] US dollars ($[**]) within [**] days of the receiving the Mid-Stage Report; and (c) [**] US dollars ($[**]) within [**] days of the receiving the Final Report.

5. Foundation Negotiation Rights. During the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, before entering into any written agreement with any third party under which PTC is obligated to conduct screening of its library for small molecules that modulate the expression of Drug Targets in exchange for funding, PTC shall first conduct good faith negotiations with Foundation with respect to provision of such funding by Foundation. Notwithstanding the foregoing, PTC's obligations under this First Amendment Section 5 shall neither prohibit nor in any way limit (a) PTC's ability to fulfill contractual commitments to third parties in effect as of the Amendment Effective Date, (b) PTC's ability to enter into license agreements or otherwise collaborate with third parties with respect to compounds or programs directed against SMA developed by such third parties; nor (c) PTC's ability to enter into any agreements or arrangements with respect to modulation of genes relevant to SMA via nonsense suppression.

6. Coordination of Funding. During the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, should PTC require additional funds for the conduct of any Research Project, the Foundation will be consulted prior to any fundraising efforts for such Research Project. Should PTC identify an opportunity for agreement with any third party or parties with respect to additional or continued funding specifically directed to Research Projects, it will provide reasonable advance notice to Foundation, and the parties will negotiate in good faith (involving such third party or parties as appropriate) to develop a structure that supports such additional funding, based on the following principles: (a) entities co-funding a Research Project should share information on the Research with each other, subject to appropriate confidentiality provisions, (b) governance with respect to co-funded Research Projects should be via a joint steering committee including representatives of Foundation, PTC, and any third parties, (c) within the steering committee for a particular co-funded Research Project, role in decision-making with respect to matters within the sole purview of funding

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entities (including but not limited to strategic discussions as outlined in section 2.4 of the Agreement) should be [**], and (d) entities that have provided funding to a co-funded Research Project should have an opportunity (subject to compliance with the terms of their respective funding agreements) to continue their support of such Research Project. For clarity, PTC's obligations under this First Amendment Section 6 shall in no way limit PTC's ability to engage in general fund-raising activities and to enter into agreements relating thereto.

7. No Conflicts. Each Party represents and covenants that (a) it has the authority and right, to enter into this First Amendment and to perform its obligations with respect to the Additional Research, and (b) during the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, it will not enter into any agreement with any third party that would conflict with the performance of its obligations hereunder, or with Foundation's potential funding of Lead Optimization on terms mutually acceptable to the parties.

8. Notices. The address of the Foundation for the purposes of section 9.7 of the Agreement shall be as follows:

Spinal Muscular Atrophy Foundation
888 Seventh Avenue
Suite 400
New York, NY 10019
Fax: 212-347-2079
Attention: Ms. Cynthia Joyce, Executive Director

With a copy to Cooley Godward LLP as currently provided in the Agreement.

9. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this First Amendment and the terms of the Agreement, the terms of this First Amendment shall govern.

IN WITNESS WHEREOF, the Parties have executed this First Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.

SPINAL MUSCULAR ATROPHY FOUNDATION

/s/ Mark E. Boulding	/s/ Loren Eng
By: Mark E. Boulding	By: Loren Eng
Title: Senior Vice President and General Counsel	Title: SMA Foundation, President

EXHIBIT A-1

RESEARCH PLAN FOR ADDITIONAL RESEARCH

The research goal of this modification to the Research Plan is to complete hit characterization of the active compounds from Project A, Project B, and Project C (the "Projects"), to perform in vitro pharmaceutical profiling of leads from these Projects, to delineate clearly a screening tier to meet the development candidate goal for these Projects, and to identify directions for the full optimization process.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [**]

Execution Version

AMENDMENT No. 2 TO SPONSORED RESEARCH AGREEMENT

This second amendment ("Second Amendment") to the Sponsored Research Agreement is entered into as of the 1st day of May, 2009 (the "Second Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007 (the "Agreement");

WHEREAS, the parties desire to further amend the Agreement to allow additional funding by Foundation in connection with continued research focused on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Second Amendment, the parties agree as follows:

1. Definitions.

(a) Section 1.5 of the Agreement ("Company Clinical Trial") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Company Clinical Trial' means any human clinical trial of a Development Candidate or Product conducted by or on behalf of Company or its Affiliates or Licensees pursuant to an effective IND submitted by or on behalf of Company or its Affiliates or Licensees."

(b) Section 1.11 of the Agreement ("Data") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Data' means all data generated as a result of the Research or as a result of Company's or its Affiliate's or Licensee's research, Development, or commercialization of Drug Candidates or Products."

(c) Section 1.15 of the Agreement ("Field") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Field' means the treatment, mitigation or prevention of [**]."

(d) Section 1.16 of the Agreement ("First Commercial Sale") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'First Commercial Sale' means the date of the first commercial sale in a country or region by or on behalf of Company or its Affiliate or Licensee of a Product to a Third Party end user in an arm's-length transaction after an NDA has been approved for such Product in such country or region."

(e) Section 1.18 of the Agreement ("IND") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'IND' means an investigational new drug application submitted for action to the FDA or any other similar application submitted for action to an appropriate Regulatory Agency in a country or group of countries other than the United States."

(f) Section 1.23 of the Agreement ("Licensee") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Licensee' means any Third Party to which Company grants rights with respect to any Lead Candidate, Reversion Candidate, Development Candidate or Product in accordance with Second Amendment Section 10."

(g) Section 1.24 of the Agreement ("Net Sales") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Net Sales' means gross amounts received by Company and its Affiliates from Third Parties other than Affiliates or Licensees for sales of a Product to Third Parties other than Affiliates or Licensees (unless such Affiliate or Licensee is the end user of such Product, in which case the amount billed therefor shall be deemed to be the amount that would be billed to a Third Party end user in an arm's-length transaction), less the following deductions, without duplication: (a) actual bad debts actually written off which are attributable to sales of such Product; (b) any rebates, quantity, trade and cash discounts, and other usual and customary discounts to customers granted and taken in the ordinary course of business; (c) retroactive price reductions, allowances, chargebacks, rebates, adjustments and amounts repaid or credited by reason of rejections or returns of such Product (including returns of such Product by reason of a product recall or damaged or defective goods); (d) freight, shipping and insurance charges; (e) distribution, packing, handling and transportation charges for Products to the extent that they are included in the price or otherwise paid by the customer; (f) compulsory payments and rebates, actually paid or deducted; (g) customs duties and other governmental charges, as well as sales, use, excise, inventory, value added, and other taxes (except income taxes), related to the sale of such Product; (h) payments, discounts, rebates, fees, reimbursements or similar payments granted to managed health care organizations or federal, state or local governments, their agencies, purchasers or reimbursers or any government subsidized programs, wholesalers or other distributors, buying groups, health insurance carriers, other institutions, or discount programs; and

(i) any write-offs from quantities of such Product donated by Company to Third Parties for charitable or humanitarian purposes, to the extent included in gross sales. The foregoing adjustments shall be consistent with customary accounting practices within Company (or its respective Affiliates) and in accordance with U.S. Generally Accepted Accounting Principles or with a similar internationally-accepted accounting standard, consistently applied."

(h) Section 1.38 of the Agreement ("Reversionary License") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Reversionary License' shall have the meaning set forth in Section 6.1(c)(2)(i) of the Agreement."

(i) Section 1.42 of the Agreement ("Third Party Patent License") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Third Party Patent License' shall have the meaning provided in Section 3.4(c)."

(j) The following defined terms shall apply as of the Second Amendment Effective Date:

"AAA" shall have the meaning set forth in Second Amendment Section 17(b).

"Appointing Party" shall have the meaning set forth in Second Amendment Section 5(h).

"Baseball Arbitration" shall have the meaning set forth in Second Amendment Section 17(a).

"Benchmark Trigger" shall have the meaning set forth in Section 3.3(b) of the Agreement.

"Buy-Out Notice" shall have the meaning set forth in Section 3.3(b)(v) of the Agreement.

"Buy-Out Right" shall have the meaning set forth in Section 3.3(b)(iv) of the Agreement.

"Chief Executive Officer" means (a) the person holding the title of Chief Executive Officer of a party at the time in question or (b) if there is no person holding the title of Chief Executive Officer of a party at the time in question, then the person holding the title of Chairman of the Board of Directors of such party at such time.

"Collaboration Activities" means direct efforts by Company or its agents to pursue any proposal related to a license, option, joint venture, collaboration, sale or other strategic transaction (other than a PTC Corporate Change) involving the DC Research or any Lead Candidate, Reversion Candidate, Development Candidate or Product, but excluding [**] entered into with a Third Party under which Company remains primarily responsible for Development and commercialization of Lead Candidates, Reversion Candidates, Development Candidates and Products. For clarity, activities routinely performed by Company's business development team to promote Company's general drug discovery and development capabilities (including discovery research in the Field) shall not constitute Collaboration Activities.

"Commercially Reasonable Efforts" means:

(a) with respect to the efforts to be expended by a party with respect to any objective, except as otherwise provided in clause (b) below, such reasonable, diligent and good faith efforts as such party [**]; and

(b) [**].

"Company Indemnitee" shall have the meaning set forth in Second Amendment Section 13(d).

"Company Losses" shall have the meaning set forth in Second Amendment Section 13(d).

"Corrective Plan" shall have the meaning set forth in Second Amendment Section 2(g)(1).

"Cost/Timeline Issue" shall have the meaning set forth in Second Amendment Section 2(g).

"DC Research" shall have the meaning set forth in Second Amendment Section 2(a).

"DC Timeline Goal" shall have the meaning set forth in Second Amendment Section 2(a).

"Development" means, with respect to a Drug Candidate, Development Candidate, or Product, all non-clinical (including preclinical) research/development, clinical research/development, and related activities directed to obtaining Regulatory Approval of such Drug Candidate, Development Candidate, or Product, including but not limited to clinical trials, toxicology studies, drug metabolism and pharmacokinetics (DMPK) studies, statistical analysis and report writing, clinical trial design and operations, preparing and submitting INDs and applications for Regulatory Approval, activities related to development and optimization of a commercial-grade manufacturing process and formulation for such Drug Candidate, Development Candidate, or Product, safety reporting, data management and all regulatory affairs and project management related to the foregoing. When used as a verb, "Develop" means to engage in Development.

"Development Candidate" or "DC" means, on a Research Project-specific basis, a Drug Candidate that the JSC formally declares meets criteria established by the JSC indicating such Drug Candidate is suitable for progression to IND-enabling pre-clinical studies in support of future human clinical trials.

"Development Deadline Document" shall have the meaning set forth in Section 3.1 of the Agreement.

"Development Election Notice" shall have the meaning set forth in Second Amendment Section 3(d).

"Development Plan" shall have the meaning set forth in Section 3.1 of the Agreement.

"Enrollees" shall have the meaning set forth in Second Amendment Section 13(b)(2).

"JSC" shall have the meaning set forth in Second Amendment Section 5(a).

"GLP Research" shall have the meaning set forth in Second Amendment Section 3(a).

"GLP Toxicology Studies" shall have the meaning set forth in Section 3.2 of the Agreement.

"Licensee Data" shall have the meaning set forth in Second Amendment Section 10(d)(ii).

"Licensee Technology" shall have the meaning set forth in Second Amendment Section 10(d)(ii).

"M&A Approval Request" shall have the meaning set forth in Second Amendment Section 9(a).

"M&A Certification" shall have the meaning set forth in Second Amendment Section 9(b)(4).

"M&A Notice" shall have the meaning set forth in Second Amendment Section 9(b).

"NDA" means a new drug application approved by the FDA or any other similar application approved by the appropriate Regulatory Agency in a country or group of countries other than the United States."

"Non-DC Research" shall have the meaning set forth in Second Amendment Section 18(a).

"Option Period" means the period commencing upon the end of the [**] day period set forth in Second Amendment Section 3(d) and ending [**] years later; provided, however that such period shall be extended for [**] if Foundation pays Company [**] US dollars ($[**]) and for a [**] if Foundation makes a [**] US dollar ($[**]) payment to Company.

"Partnering Notice" shall have the meaning set forth in Second Amendment Section 10(d).

"Patients" shall have the meaning set forth in Second Amendment Section 13(c)(1).

"[**]" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Phase 1 Clinical Trial" means any human clinical study of a Product that is intended as initial clinical safety testing in healthy volunteers or a limited patient population, or studies directed toward understanding the mechanisms or metabolism of the Product.

"Phase 2 Clinical Trial" means any human clinical study of a Product subsequent to a Phase 1 Clinical Trial and prior to a Pivotal Clinical Trial that is intended to study the safety, dosage and initial efficacy in a limited patient population, and is prospectively designed to support the continued testing of the Product in one or more further Phase 2 Clinical Trials or in a Pivotal Clinical Trial.

"Pivotal Clinical Trial" means a pivotal human clinical study of a Product that is prospectively designed to confirm with statistical significance in an expanded patient population the efficacy and safety of a drug in a given patient population, and the results of which are intended to form the basis for Regulatory Approval. For the avoidance of doubt, a clinical trial that meets the foregoing criteria shall be deemed a Pivotal Clinical Trial regardless of whether it is characterized as a "Phase 2b," or "Phase 2b/3," or "Phase 3" clinical trial.

"Proof-of-Concept" means, with respect to a particular Development Candidate, (a) the initiation of a Pivotal Clinical Trial for the treatment, mitigation or prevention of SMA or, with the written consent of the Foundation, any other disease, indication or medical condition or (b) if sooner, the submission of an application for Regulatory Approval for the use of such Development Candidate to treat, mitigate or prevent SMA or, with the written consent of the Foundation, any other disease, indication or medical condition.

"Proposals" shall have the meaning set forth in Second Amendment Section 17(d).

"[**]" shall have the meaning set forth in Section 3.4(a) of the Agreement.

"PTC Corporate Change" means (a) a merger, consolidation, amalgamation, share exchange, business combination, issuance of securities (other than Company's initial public offering registered on Form S-1 (or any successor form) under the Securities Act of 1933, as amended, and the rules promulgated thereunder), acquisition of securities, reorganization, recapitalization, tender offer, exchange offer or other similar transaction as a result of which either (i) Company's stockholders immediately prior to such transaction in the aggregate cease to own at least 50% of the voting shares of the entity surviving or resulting from such transaction (or the ultimate parent entity thereof) (where voting refers to being entitled to vote for the election of directors or similar management body of the applicable entity) or (ii) in which a Third Party or "group" (as defined in the Securities Exchange Act of 1934, as amended, and the rules promulgated thereunder) (excluding a "group" consisting of existing stockholders of Company as of the date of this Agreement) directly or indirectly acquires beneficial or record ownership of securities representing 50% or more of Company's voting shares or (b) a sale, lease, exchange, transfer, license, acquisition or disposition of at least 50% of the assets of Company and its subsidiaries, taken as a whole, in a single transaction or a series of related transactions. For purposes of clarity, a "reverse merger," in which in a transaction or series of related transactions, Company consolidates or merges with another entity and the holders of the outstanding voting shares of Company immediately preceding such consolidation or merger hold more than fifty percent (50%) of the voting shares of the resulting entity, shall not be considered to be a PTC Corporate Change.

"PTC License Income" means all royalties, license fees, milestone payments, annual maintenance fees or similar payments or consideration paid by a Licensee to Company or its Affiliates in consideration for the grant by Company or its Affiliate of a license to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidates or Products or for the practice of such license (with any of the foregoing

consideration received by Company other than in the form of cash to be valued at its fair market value as of the date of receipt), provided that PTC License Income shall exclude the proceeds of any debt or equity issuance (except to the extent such payments exceed the fair market value of such securities upon date of receipt, in which event such excess over fair market value shall be included in the calculation of PTC License Income), research and development funding (except to the extent such funding is not reimbursement for the Company's commercially reasonable out-of-pocket, personnel and indirect expenses incurred after the grant of such license to such Licensee and pursuant to a research or development plan approved by such Licensee, in which event such excess shall be included in the calculation of PTC License Income), and any merger or acquisition consideration.

"Publishing Party" shall have the meaning set forth in Section 5.4(a) of the Agreement.

"Regulatory Agency" means, with respect to the United States, the FDA, and, in the case of a country other than the United States, such other appropriate regulatory agency or authority with similar responsibilities.

"Repayment Amount" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Research Cap" shall have the meaning set forth in Second Amendment Section 2(d).

"Research Compound" shall have the meaning set forth in Second Amendment Section 18(c).

"Research Report" means (a) with respect to any report made prior to the Second Amendment Effective Date, a report defined as such in Section 2.7 of the Agreement or a report made pursuant to First Amendment Section 3, or (b) with respect to any report made on or after the Second Amendment Effective Date, a report described in Second Amendment Section 4(b).

"Reversion Candidate" means (a) each Development Candidate and (b) each Lead Candidate designated as such pursuant to Second Amendment Section 5(b)(vi) or 5(c)(i).

"Reversion Notice" means a notice identified as such in Section 3.2, 3.4(a)(i), 3.4(b), 3.4(c), 3.4(d), or 6.1(c)(1) of the Agreement or Second Amendment Section 3(d).

"Reversion Products" shall have the meaning set forth in Section 6.1(c)(2)(i) of the Agreement.

"Reviewing Party" shall have the meaning set forth in Section 5.4(a) of the Agreement.

"Sales Threshold" shall have the meaning set forth in Section 4.3(b) of the Agreement.

"Second Amendment Term" means the period from the Second Amendment Effective Date until the end of the Research Term.

"Secondary Research Project" shall have the meaning set forth in Second Amendment Section 2(a).

"SMAF Clinical Trials Advisory Committee" shall have the meaning set forth in Second Amendment Section 13(a).

"SMAF Funding Amount" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Special Termination" shall have the meaning set forth in Second Amendment Section 3.

"Term" shall have the meaning set forth in Section 7.1 of the Agreement.

"Worldwide Net Sales" means the sum of (i) Net Sales and (ii) net sales by Licensees, with such net sales being calculated according to the definition of "Net Sales," but substituting "Licensee" for "Company" as the context requires; provided, however, that if pursuant to a written license agreement with Licensee, Company has agreed to a commercially reasonable definition of net sales by such Licensee that is reported to Company by Licensee on a quarterly basis, such reported net sales may be used in the calculation of "Worldwide Net Sales."

(k) Except as expressly set forth herein, all capitalized terms used herein and not otherwise defined shall be as defined in the Agreement. For clarity, all definitions of terms that

reference Agreement Sections refer to those Agreement Sections as amended by this Second Amendment.

2. Continuing Research.

(a) The parties agree to the modification of the Research Plan and related budget attached as Exhibit SA-1 to allow for Company to perform continued research activities (the "DC Research") with respect to [**] Research Projects previously funded under the Agreement and the First Amendment. The goal of such research will be the presentation by Company of one (1) Development Candidate from one (1) Research Project for further discussion with Foundation with respect to potential funding of development of such Development Candidate by Foundation, and continuation of one (1) backup program with respect to the other Research Project (the "Secondary Research Project"). The expected duration of the DC Research is [**] months from the Second Amendment Effective Date (the "DC Timeline Goal").

(b) Company shall conduct such DC Research (i) in accordance with Exhibit SA-1, subject to amendment by the JSC as provided in this Second Amendment, and (ii) in accordance with the terms of the Agreement as amended herein, including but not limited to Company's obligations under Section 2.5 of the Agreement (captioned "Performance Standards").

(c) In connection with such DC Research, the Research Term shall be extended, without interruption, until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) the date which is [**] years following the Second Amendment Effective Date or (iii) the effective date of any

termination of the Research Term pursuant to Second Amendment Section 3.

(d) The parties will fund the overall total cost of the DC Research based on the Research Plan and related budget attached as Exhibit SA-1, with Foundation contributing approximately [**]% and Company contributing approximately [**]% of such overall total cost of the DC Research as more explicitly specified in such budget, such overall total cost not to exceed $[**] (the "Research Cap") and the Foundation's share of such total cost not to exceed $[**]. During the Research Term, Company will invoice Foundation on a quarterly basis for Foundation's share of the costs incurred in connection with the Research Plan for the preceding calendar quarter, payable within [**] days of receipt by Foundation, subject to Second Amendment Sections 2(d)(i) and 2(d)(ii). Such invoices shall include: (A) an accounting, in reasonable detail sufficient to evaluate performance of the Research Plan by Company, of Company's activities over the applicable period, (B) a breakout of FTEs and other resources allocated to each Research Project and (C) an itemization in reasonable detail of the categories of out-of-pocket costs incurred by Company that are included in such invoice. When invoicing Foundation or developing or presenting any budget related to the Research Plan, Company will in all cases apply the FTE rates specified in Exhibit SA-1 to the applicable category of FTE, and no additions or changes to the FTE categories or rates specified in Exhibit SA-1 shall be made by Company absent prior written consent of Foundation. Company will promptly respond to all requests by Foundation for additional information regarding such out-of-pocket costs. Company's commitment, between [**] and [**], of [**] dollars ($[**]) in funding towards the DC Research shall be available to Company in the form of an invoice credit against Company's share of the cost of the DC Research until expended and shall count towards the Research Cap.

Promptly after the Second Amendment Effective Date, Company will provide Foundation with an invoice for [**] percent ([**]%) of the amount that Company spent between [**] and the Second Amendment Effective Date to perform the DC Research. Such invoice shall include the information specified in (A), (B) and (C) within this Second Amendment Section 2(d) and shall be payable within [**] days of receipt by Foundation. The entire amount paid by Foundation pursuant to such invoice shall count towards the Research Cap and towards Foundation's share of the Research Cap.

(i) Subject to Second Amendment Section 2(d)(ii), Foundation shall not be responsible for its share of any DC Research costs that exceed the budget for any calendar quarter unless:

(1) such costs exceed the budget for such calendar quarter by less than [**] dollars ($[**]) or [**] percent ([**]%) (whichever is less);

(2) such costs exceed the budget for such calendar quarter by more than [**] dollars ($[**]) or [**] percent ([**]%) (whichever is less) but less than [**] percent ([**]%) and Company provided written notice to Foundation prior to incurring such budget overrun; or

(3) such costs exceed the budget for such calendar quarter by more than [**] percent ([**]%) and Foundation approved such budget overrun in writing before it was incurred.

(ii) If at any time during the Second Amendment Term, the total cost incurred in the performance of the DC Research during the period from the Second Amendment Effective Date until the end of the most recent calendar quarter exceeds the cumulative budget for such period by [**] dollars ($[**]) or more, then Second Amendment Section 2(d)(i) shall not apply to any subsequent cost overruns and Foundation shall not be responsible for its share of any additional costs that exceed the applicable budget for any subsequent quarter unless Foundation approved such budget overrun in writing before it was incurred.

(e) Foundation may provide its share of the budget under the Research Plan via other sources of funding, subject to prior agreement of the parties and the existing terms of the Agreement. One hundred percent (100%) of all funds, if any, received by Company during the Second Amendment Term from the Department of Defense directed to the DC Research or the Development of a Development Candidate as a result of the advocacy of the Foundation will count toward the Foundation's share of the costs incurred in connection with the Research Plan; provided, however, that [**] in the [**] pursuant to [**] of the Agreement. At Foundation's request, Company shall promptly complete all paperwork required or reasonably useful to secure receipt by Company of such funds from the Department of Defense.

(f) Company may provide its share of the budget via government grants or grants from nonprofit organizations; provided however, that, except for mandatory licenses and similar or related rights granted to government entities, Company's acceptance of such grants shall not have any effect on Foundation's rights pursuant to this Agreement; further provided, however, that with respect to any nonprofit organizations that have as a specific aspect of their general

mission the funding of research on SMA, Company shall first seek Foundation's written consent and the parties shall negotiate in good faith any required amendments to this Agreement or separate agreements to accommodate grants from such organizations, with the guiding principle that this Agreement remain the primary document governing the conduct of the DC Research by the parties. Company shall use Commercially Reasonable Efforts to obtain additional funding for the Secondary Research Project from government grants or grants from nonprofit organizations (subject to the provisions set forth in the preceding sentence with respect to funding from any nonprofit organizations that have as a specific aspect of their general mission the funding of research on SMA); provided however, that, except for mandatory licenses and similar or related rights granted to government entities, Company's acceptance of such grants shall not have any effect on Foundation's rights pursuant to this Agreement. If Company obtains such funding in an amount that exceeds Company's share of the budget for the Secondary Research Project, Company shall notify Foundation in writing and the JSC shall revise the Research Plan and related budget to reflect the additional work to be performed on the Secondary Research Project with such funds, (i) which additional work shall be under the purview of the JSC and the compounds resulting from such work shall remain Drug Candidates, Reversion Candidates, or Development Candidates, as the case may be, and (ii) which revised budget shall not require the Foundation to contribute any money to pay for or reimburse Company for research performed with respect to any aspect of the revised Research Plan for which Company has received such grant funds.

(g) If (i) it becomes evident to either party at any time, based on budget forecasts or progress in the Research Plan, that a [**] may [**] for [**], or that the [**], or (ii) the [**] is [**] (each of the foregoing, a "Cost/Timeline Issue"), then either party may, on written notice to the other, call a special meeting of the JSC to address such Cost/Timeline Issue. At such meeting, representatives of each party shall present information in their control with respect to the reasons for such Cost/Timeline Issue, and (if applicable) each party's plan or recommendation for addressing such Cost/Timeline Issue. The JSC shall review and address such Cost/Timeline Issue, and shall determine which of the following actions the parties shall pursue:

(1) develop, approve, and follow an amendment to the Research Plan (such amendment, the "Corrective Plan") to address the Cost/Timeline Issue, which may (subject to the written consent of the affected party in such party's sole discretion) require either party to [**] the DC Research, or provide that the [**] in which case (x) the [**] shall be [**] and/or, if the [**] is more than [**] years after the Second Amendment Effective Date, then the

Research Term shall be deemed amended to extend until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) the [**], or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3, and (y) in addition to their other obligations under the Agreement, the parties shall duly perform their respective obligations pursuant to such Corrective Plan; provided, however, that after the adoption of a Corrective Plan, failure to achieve the [**] or [**] shall not be deemed, by itself, to be a breach of this Agreement, but shall entitle either party to terminate the Research Term pursuant to Second Amendment Section 3;

(2) restructure the Research Plan and related budget in a manner that resolves the Cost/Timeline Issue; provided, however, that such restructuring shall not obligate either party to [**] of the [**] or be deemed to [**]; or

(3) determine that continuation of the DC Research would be futile, in which case the JSC shall recommend to the parties that they terminate the DC Research; provided further, that following such recommendation either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3.

(h) If the members of the JSC fail to unanimously agree upon one of the three actions described in Second Amendment Section 2(g) (1), (2) and (3), then the matter shall be referred to the parties' Chief Executive Officers, and if the parties' Chief Executive Officers do not agree upon one of such three actions within [**] days after matter referral, then either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3. If the affected party does not approve the Corrective Plan within [**] days after it is first formally proposed, then either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3.

3. Special Termination. In addition to the rights to terminate this Agreement as provided in Article 7 of the Agreement, either party shall have the rights to terminate the Research Term as provided in Second Amendment Section 2(g)(1), 2(g)(3) or 2(h) (any such termination of the Research Term, a "Special Termination"). Upon written notice from one party to the other party consistent with the provisions of Second Amendment Section 2(g)(1), 2(g)(3) or 2(h) and specifically identifying the circumstances giving rise to a right of Special Termination, a Special Termination shall go into effect and neither party shall have any rights or obligations with respect to the other party pursuant to this Agreement except as specifically set forth in this Second Amendment Section 3. Upon the effectiveness of a Special Termination:

(a) subject to Second Amendment Section 3(b), Foundation shall automatically have a worldwide, fully-paid up and royalty-free, nonexclusive, nontransferable (except in connection with the assignment of this Agreement pursuant to Section 9.1 of the Agreement), sublicensable (solely as set forth in this Second Amendment Section 3(a)) right (i) under the Company Technology, Licensee Technology, Data, Licensee Data and Company Base IP, to make, have made and import Reversion Candidates and to use Reversion Candidates for its own internal purposes and for pre-clinical research activities ([**] any pre-clinical research performed under good laboratory practice guidelines (such pre-clinical research, "GLP Research")) in the Field, (ii) to access or reference any filings made by Company or its agents with Regulatory Authorities with respect to any Reversion Candidate, (iii) to receive within [**] months of the effectiveness of the Special Termination copies of all Data and Licensee Data and all reports and other information that were (or should have been) accessible to Foundation prior to the Special Termination via the shared electronic collaboration space described in Second Amendment Section 4(a), (iv) to receive within [**] months of the effectiveness of the Special Termination reasonable quantities of existing stock of materials (other than (1) materials that are [**] or (2) materials that [**]) in Company's possession or under its control and (xx) that are specific to, or were used or were contemplated to be used in, the DC Research, and are not commercially available from Third Parties, (yy) that are reasonably necessary for continued research or

preclinical testing of Reversion Candidates or were used in, or were contemplated to be used, in the DC Research, and (zz) the transfer of which would not infringe any Third Party intellectual property rights (and no non-infringing alternative is identified after a reasonable inquiry), trigger a breach of any contractual obligations of Company with respect to a Third Party (other than a Licensee), or [**] trigger any contractual obligation to make payments to a Third Party (other than a Licensee); provided, however, that any subsequent transfers of such materials by Foundation to Third Parties shall be subject to the terms of a materials transfer agreement reasonably acceptable to Company, and (v) to gain access [**] to reasonable quantities of Company's existing stock of Reversion Candidates for its own internal purposes and for pre-clinical research activities ([**] any GLP Research) in the Field; such right to be sublicensable by Foundation to (1) a contract research organization or non-academic Foundation collaborator only upon prior written notice to Company or (2) an academic or governmental Foundation collaborator only with the prior written consent of Company, such consent not to be unreasonably withheld or delayed and only to be withheld based on objective criteria determined by the JSC within [**] months after the Second Amendment Effective Date. At Foundation's request and expense, Company shall provide Foundation with reasonable assistance to facilitate Foundation's practice of the foregoing right, including disclosure of Company Know-How, provision of technical assistance and facilitation of Foundation's efforts to obtain supply of Reversion Candidates from the Third Party who supplied such Reversion Candidate to Company prior to the Special Termination;

(b) each party shall keep the other party reasonably informed with respect to the results of any non-clinical, pre-clinical research ([**] GLP Research) and clinical testing performed upon any Reversion Candidate by or on behalf such party following a Special Termination (which clinical testing and GLP Research, if in the Field, shall only be performed after the obligations set forth in Second Amendment Section 3(c) have been satisfied);

(c) neither party may perform upon, any Reversion Candidate, any clinical testing in the Field or any GLP Research in the Field without first providing written notice to the other party and [**];

(d) if either party provides the other party with notice and [**] pursuant to Section 3(c) of this Second Amendment but the parties do not, within [**] months after such notice, [**] with respect to the [**], then Company may within the next [**] days provide written notice (a "Development Election Notice") to Foundation stating that Company intends to pursue continued Development and commercialization of one or more Reversion Candidates in the Field using Commercially Reasonable Efforts and identifying the Reversion Candidate of greatest interest to Company; such Reversion Candidate shall be deemed to be a Development Candidate selected by the JSC as of the date of the Development Election Notice. If Company provides a Development Election Notice within such [**] day period, then, notwithstanding any other provision of this Second Amendment Section 3, the parties shall have all rights and obligations under this Agreement that apply to periods after the end of the Research Term and the JSC shall resume functioning as specified in Second Amendment Section 5(a). If Company does not provide a Development Election Notice within such [**] day period, then upon Foundation's written notice to Company (such notice, a "Reversion Notice") within the Option Period,

Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement;

(e) notwithstanding any other provision of the Agreement to the contrary (except for Second Amendment Section 3(d)), only the provisions of Sections 4.7, 6.1(a), 6.1(b), 6.1(c)(2), 6.1(c)(4), 6.2, 7.5 of the Agreement, and Articles 1, 5, 8, and 9 of the Agreement, Sections 5 and 6 of the First Amendment (*provided*, that the [**] month periods referenced in Sections 5 and 6 of the First Amendment shall terminate [**] months after the effectiveness of the Special Termination), and Sections 1, 3, 4(b)(ii), and 18(a) of this Second Amendment will survive such Special Termination; *provided*, *however*, that in the event Foundation subsequently obtains a Reversionary License pursuant to Second Amendment Section 3(d), then all provisions of this Agreement will continue to apply except to the extent terminated pursuant to Section 6.1(c)(2)(vi) of the Agreement; and

(f) except as explicitly set forth in Second Amendment Section 3(a) with respect to certain optional costs payable by Foundation, Foundation shall not have any obligations to pay for any research-associated costs incurred after the effective date of the Special Termination.

4. Research Reports and Access to Information. In lieu of the Research Reports and other information and communications that would otherwise be due from Company under Sections 2.4 and 2.7 of the Agreement during the Research Term, or pursuant to Section 3 of the First Amendment, Company shall make the following reports and information available:

(a) Information. Promptly after the Second Amendment Effective Date, Company will establish a shared electronic collaboration space that enables designated representatives of Foundation to access and provide information on the progress of the DC Research. For clarity, the persons listed on Exhibit SA-5 of this Second Amendment are, as of the Second Amendment Effective Date, designated representatives of Foundation for such purpose. Foundation may remove any such designated representative at any time upon written notice to Company. Foundation may also appoint new designated representatives subject to the conditions specified in Second Amendment Section 18(f). Such information shall include agendas and minutes of team meetings, presentations, correspondence between the parties, and data and reports from the DC Research, as well as monthly FTE reports (which reports shall be posted no later than [**] days after the end of the applicable month and shall list the number of hours that each person (identified by name and general job description (e.g., "chemist")) worked on the DC Research during such month). Company shall post data from the ongoing conduct of the DC Research to such electronic collaboration space on a regular and continuing basis; provided, that (i) the frequency of such posting may be adjusted by consent of the JSC, and (ii) in the absence of any such consent, Company shall post such data at the same time and in the same format as made available to Company's internal project leadership team (a sample of which format is appended as Exhibit SA-2). Company shall have the right to limit access to sensitive data (by way of example, but not limited to, non-public chemical structures) to a mutually-agreeable list of representatives of Foundation. Such list, as of the Second Amendment Effective Date, is set forth on Exhibit SA-6. Foundation may remove any such representative from such list at any time upon written notice to Company. Foundation may also add new representatives to such list subject to the conditions specified in Second Amendment Section 18(f).

(b) Reports. (i) Within [**] days of the end of each [**] or at least [**] prior to any [**] meeting of the JSC (whichever comes first), or such other regular times as the parties may otherwise agree, Company shall provide to Foundation with a reasonably detailed written summary report of the results (including Company's analysis thereof) and progress of the DC Research during such [**] and expectations for DC Research to be conducted during the immediately subsequent [**], and (ii) within [**] days of completion of the DC Research or termination of the DC Research on account of a Special Termination or pursuant to Article 7 of the Agreement, Company shall provide to Foundation a final report summarizing the status and accomplishments of the DC Research and containing the recommendations by Company with respect to selection of a Development Candidate with respect to one Research Project and for further research towards a potential Development Candidate with respect to the other Research Project. Company will promptly provide all information reasonably requested by Foundation regarding the DC Research described in any report provided pursuant to this Section 4(b) of this Second Amendment.

(c) Availability for Communications. In addition to the foregoing and to Company's obligations under Section 2.5 of the Agreement, Company will make appropriate representatives of the scientific team conducting the DC Research available for conference calls and meetings with appropriate representatives of Foundation at reasonable times and places for informal discussion of the progress of the DC Research. In further addition, the Foundation may, at its option, during the Term, schedule up to [**] formal program review meetings with Company personnel and those of Foundation's Third Party advisors who (i) have been designated by Foundation in compliance with Second Amendment Section 18(f), and (ii) are reasonably acceptable to Company. Such meetings will be held at the times and locations mutually agreed upon by the parties. The purpose of such meetings will be to review the progress of the Research relative to the Research Plan.

5. Governance. The parties agree to the following provisions with respect to governance of their collaboration:

(a) Joint Steering Committee. The parties will establish a joint steering committee ("JSC") consisting of equal representation from Foundation and Company within [**] days after the Second Amendment Effective Date. The parties acknowledge and agree that the individuals listed on Exhibit SA-8 have been approved, as of the Second Amendment Effective Date, to serve as the Foundation's representatives to the JSC and there is no need for the parties to perform the procedures set forth in Second Amendment Section 18(f) with respect to their appointment to the JSC. The JSC shall be comprised of at least [**] representatives of each party, each with appropriate decision-making authority to enable the JSC to fulfill its obligations under this Agreement, and which in the case of Foundation may be Third Party advisors of Foundation, provided they are appointed pursuant to the conditions specified in Second Amendment Section 18(f). Changes in the designation of JSC members by each party may occur at any time during the Term upon written notification by a party to the other party. The JSC, as its first order of business, shall select a chairperson from one party and a secretary from the other party, to alternate on an annual basis. Subject to the confidentiality provisions of the Agreement and any appropriate agreements with respect to intellectual property or conflicts of interest, the JSC may invite other representatives of the parties with special skills or knowledge (and who, in

the case of Foundation, may be Third Party advisors of Foundation) to attend JSC meetings where appropriate. Each party shall disclose to the other its proposed agenda items in advance of each JSC meeting, and the chairperson shall distribute a draft agenda reflecting such proposed agenda items reasonably in advance of each meeting. The JSC shall adopt such other procedural rules as are necessary or convenient for its work. Each party shall be responsible for all travel and other costs for its representatives to attend meetings of, and otherwise participate on, the JSC. The JSC shall continue to function until the earliest of: (i) the effective date of a Special Termination, (ii) the Company's receipt of a Reversion Notice or a Buy-Out Notice or (iii) the end of the Term. If the JSC stopped functioning on account of a Special Termination and the Company subsequently provides a Development Election Notice pursuant to Second Amendment Section 3(d), then the JSC shall resume functioning promptly upon the Foundation's receipt of such Development Election Notice. Such reconvened JSC shall have the duties specified

in Second Amendment Section 2(c) and, regardless of whether Proof-of-Concept has been achieved as of the date of the Development Election Notice, it shall meet and make decisions in accordance with the provisions of Second Amendment Section 5(e) (and not Second Amendment Section 5(d)).

(b) Duties of the JSC during the Research Term. During the Research Term, the JSC shall be responsible for:

(i) monitoring the parties' activities under the Research Plan and the Agreement;

(ii) reviewing and approving amendments to the Research Plan (and related budget), and at least once each calendar year formally reviewing and updating the Research Plan (and related budget) on a comprehensive basis;

(iii) in connection with the review and approval of the Research Plan (and related budget) and any amendments thereto, identifying appropriate resources necessary to conduct the DC Research and adjusting, as necessary to further the purpose of the DC Research, the budget for the Research Plan;

(iv) establishing timelines and criteria for continuation/discontinuation decision points under the DC Research;

(v) establishing and revising minimum activity and safety criteria for Lead Candidates from each Research Project within the DC Research (which criteria may be different for the [**] Research Projects within the DC Research);

(vi) maintaining and updating at each JSC meeting during the Research Term, one list for each of the [**] Research Projects within the DC Research that identifies and rank orders all potential and actual Lead Candidates and Development Candidates from such Research Project and denotes all Development Candidates and between [**] and [**] potential or actual Lead Candidates from such Research Project as "Reversion Candidates";

(vii) deciding whether to pursue (1), (2) or (3) of Section 2(g) of this Second Amendment in the event of a Cost/Timeline Issue;

(viii) establishing criteria for, and designating, Development Candidate(s);

(ix) providing a forum for discussion/presentation regarding, and serving as the sole governance body for decision-making regarding, research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s); for clarity the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision;

(x) prior to the designation of a Development Candidate, preparing the Development Plan for such Development Candidate and reviewing and updating the Development Deadline Document as it may deem advisable, in each case as further provided in Article 3 of the Agreement;

(xi) serving in the role specified in Second Amendment Section 10 with respect to transactions arising in connection with Collaboration Activities;

(xii) establishing policies and procedures governing scientific publications and presentations, and if the JSC deems it advisable, establishing a publication committee to administer such policies and procedures, as further provided in Section 5.4(a) of the Agreement;

(xiii) except for those rights and obligations specified in Section 4 of this Second Amendment, serving in lieu of the parties with respect any rights or obligations to review, communicate, inform, meet or discuss otherwise provided for in Sections 2.2, 2.4, and 2.7 of the Agreement;

(xiv) developing the criteria specified in Second Amendment Sections 3(a) and 18(f) within [**] months of the Second Amendment Effective Date;

(xv) reviewing scientific and medical literature to identify diseases, indications or medical conditions that, [**] or [**], are [**] for [**] and [**] diseases, indications or medical conditions [**];

(xvi) performing those other tasks specifically allocated to it in this Agreement that are applicable during the Research Term; and

(xvii) otherwise serving as a forum for exchanging information and discussing the progress of the collaboration between Company and Foundation pursuant to the Agreement.

(c) Duties of the JSC Following the Research Term. Following the Research Term, the JSC shall be responsible for:

(i) at the first JSC meeting after the end of the Research Term, (1) reviewing each potential or actual Lead Candidate that was not designated as a Development Candidate

during the Research Term and either designating it as a Development Candidate or determining that it does not meet the criteria for designation as a Development Candidate and (2) preparing a final list (which can only be subsequently changed by the written agreement of the parties) for each of the [**] Research Projects within the DC Research that identifies and rank orders all potential and actual Lead Candidates and Development Candidates from such Research Project and denotes all Development Candidates and between [**] and [**] potential or actual Lead Candidates from such Research Project as "Reversion Candidates";

(ii) following the designation of a Development Candidate, and at least [**] thereafter, conducting a formal review and comprehensive update of the Development Plan and Development Deadline Document for such Development Candidate, in each case as further provided in Article 3 of the Agreement;

(iii) monitoring Company's and its Affiliates and Licensees activities with respect to the Development Plan and Development Deadline Document;

(iv) providing a forum for discussion/presentation regarding, and serving as the sole governance body for decision-making regarding, Development, commercialization, and Collaboration Activities with respect to Reversion Candidates, Development Candidate(s) and Product(s); for clarity the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding Development, commercialization, and Collaboration Activities with respect to Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision;

(v) serving in the role specified in Second Amendment Section 10 with respect to transactions arising in connection with Collaboration Activities;

(vi) establishing policies and procedures governing scientific publications and presentations, and if the JSC deems it advisable, establishing a publication committee to administer such policies and procedures, as further provided in Section 5.4(a) of the Agreement;

(vii) except for those rights and obligations specified in Section 4 of this Second Amendment, serving in lieu of the parties with respect any rights or obligations to review, communicate, inform, meet or discuss otherwise provided for in Sections 2.2, 2.4, and 2.7 of the Agreement;

(viii) reviewing scientific and medical literature to identify diseases, indications or medical conditions that, [**] or [**], are [**] for [**] and [**] diseases, indications or medical conditions [**];

(ix) performing those other tasks specifically allocated to it in this Agreement that are applicable after the Research Term; and

(x) otherwise serving as a forum for exchanging information and discussing the progress of the collaboration between Company and Foundation pursuant to the Agreement.

(d) Meetings and Decision-Making by the JSC — Before Proof-of-Concept. During the Research Term and through achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**], in person (with locations to alternate between the parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or the secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall meet in person to attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment; provided, however, that the following matters shall not be subject to such referral to Baseball Arbitration, and any disputes arising in the JSC with respect to them may only be resolved by mutual agreement of the parties: (i) [**]; (ii) any [**] described in Second Amendment Section [**]; (iii) any changes to the [**] that would require [**] than contemplated in the [**]; and (iv) deciding whether to pursue ([**] of this Second Amendment in the event of a [**].

(e) Meetings and Decision-Making by the JSC — Following Proof-of-Concept. Following achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**] during each calendar year, in person (with locations to alternate between the parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then [**] shall have the deciding vote on the matter; provided, however, that the following matters shall not be subject to such [**] final determination, and any disputes arising in the JSC with respect to them may only be resolved as set forth below: (i) any [**], and (ii) the [**] with respect to [**] in connection with [**] set forth in, and subject to, Second Amendment Section [**].

(f) Meeting Minutes. The secretary (or if absent, such acting secretary as the chairperson shall designate) shall be responsible for preparing the minutes of the JSC meeting. Such JSC meeting minutes shall provide a description in reasonable detail of the discussions held at the meeting, and a list of any actions, decisions or determinations made by the JSC. Unless otherwise agreed by the JSC, the secretary shall distribute draft minutes of each meeting within

[**] days after the meeting for review and comment, and final minutes shall be approved by both parties within [**] days after the meeting.

(g) Joint Teams. Within [**] days after the Second Amendment Effective Date, the JSC shall establish a Joint Team with appropriate representation from the parties, which in the case of the Foundation may be Third Party advisors of Foundation appointed pursuant to the conditions specified in Second Amendment Section 18(f), to assist the JSC in the execution of the Research Plan. The parties acknowledge and agree that the individuals listed on Exhibit SA-8 have been approved, as of the Second Amendment Effective Date, to serve as the Foundation's representative to the Joint Team and there is no need for the parties to perform the procedures set forth in Second Amendment Section 18(f) with respect to their appointment to the Joint Team. The JSC shall have the authority to establish one or more additional Joint Teams with appropriate representation from the parties to assist the JSC in the performance of its duties. The JSC may establish such procedural rules and meeting schedules for such Joint Teams as it deems appropriate; provided, that unless otherwise agreed by the JSC each Joint Team shall meet at least [**], and shall report on its activities to the JSC at regularly-scheduled JSC [**] meetings. The JSC may change the composition of any Joint Team at any time upon notice to the parties.

(h) Appointment of JSC Members and Joint Team Members. The appointment of members of the JSC and any Joint Team is a right of each party and not an obligation and shall not be a "deliverable" as defined in EITF Issue No. 00-21. Each party shall be free to determine not to appoint members to the JSC

and any Joint Team, and at any time during the Term and for any reason, either party shall have the right to withdraw from participation in the JSC and any Joint Team upon written notice to the other party, which notice shall be effective immediately upon receipt. If a party ("Appointing Party") does not appoint members of the JSC or any Joint Team, or withdraws from the JSC or any Joint Team, it shall not be a breach of this Agreement, nor shall there be any associated penalty due nor shall there be any impact on the consideration otherwise provided for or due to the Appointing Party under this Agreement, and unless and until such persons are again appointed: (i) the other party, without regard to the provisions of this Second Amendment Section 5 with respect to voting, quorum or dispute resolution, may discharge the roles of the JSC and any Joint Team for which appointments were not made or with respect to which a withdrawal or removal has occurred by the Appointing Party (including designating a chairperson and secretary of the JSC and making all decisions within the decision-making authority of the JSC, which decisions shall be binding thereafter on both parties) and (ii) where the Appointing Party has not made appointments to the JSC or has withdrawn from the JSC, the Appointing Party shall not participate in any meetings of the JSC and shall not have the right to approve the minutes of any JSC meeting. If, at any time following the Second Amendment Effective Date, a party has not appointed or has pursuant to this Second Amendment Section 5(h) withdrawn from the JSC or any Joint Team, and such party wishes to resume participating in the JSC or any Joint Team, such party shall notify the other party in writing and, thereafter, such notifying party's designees shall be entitled to attend any subsequent meeting of the JSC or any Joint Team and to participate in the activities of, and decision-making by, the JSC or any Joint Team, in each case as provided in this Second Amendment Section 5 as if a failure to appoint or submitting the withdrawal notice had not occurred.

6. Information Concerning other SMA Efforts. The parties acknowledge that a goal of Foundation in funding the DC Research is to identify and advance the compound most likely to advance rapidly to human clinical trials directed towards the treatment, mitigation or prevention of SMA, and that therefore the parties may have an interest in negotiating funding of other research and development efforts conducted by Company instead of, or in addition to, the Research Projects. In furtherance of this objective, Company will make available to Foundation on a confidential basis regular reports with respect to progress and summary data with respect to Company's other internal efforts directed towards the approval of a compound for the treatment, mitigation or prevention of SMA. In addition, Company will make available to Foundation general product profiles showing, on a comparative basis, the status of potential Development Candidates from the DC Research against other potential therapeutic agents being pursued by Company in the treatment, mitigation or prevention of SMA (whether internal or in collaboration with Third Parties) in the format provided in Exhibit SA-3 to this Second Amendment; provided, however, that such obligation shall not require Company to breach any condition of any agreement in effect as of the Second Amendment Effective Date. Company will use Commercially Reasonable Efforts to ensure that it is able to share the information specified in this Second Amendment Section 6 with respect to any Third Party agreements entered in to following the Second Amendment Effective Date, and may only enter into such Third Party Agreements if it notifies Foundation reasonably in advance of entering in to such agreements to allow further discussions and potential negotiations with such Third Party with respect to such sharing of information. If, based on information made available pursuant to this Second Amendment Section 6, either party is of the opinion that a change in funding or approach may be advisable, then such party may propose to the JSC, and the JSC shall conduct, an evaluation of the merits of such proposed change that includes a report and recommendation thereon to the parties.

7. Foundation Access to Company Meetings Following Declaration of a Development Candidate. Following the JSC's determination that a particular compound is a Development Candidate and through the earlier of Regulatory Approval, abandonment of Development of such Development Candidate, or the granting of a Reversionary License to Foundation, Company shall invite a representative of Foundation (to be designated by Foundation) to observe regularly scheduled monthly meetings of the Company team charged with Development of such Development Candidate, subject to the terms of Second Amendment Section 18(f); provided, however, that failure of Foundation to designate such representative or failure of such representative to attend such meetings shall not constitute a breach of this Agreement. Company may request that Foundation representative recuse themselves from such meetings (or portions of such meetings) (a) that do not relate specifically to a Development Candidate, (b) to prevent the breach of an applicable legal or regulatory obligation of confidentiality or privacy or avoid a conflict of interest, (c) to protect the attorney-client privilege, and/or (d) to preserve intellectual property rights.

8. Development of Products.

(a) Article 3 of the Agreement (captioned "Development of Products") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"**3. DEVELOPMENT OF PRODUCTS.**

"3.1 Development Plan and Development Deadline Document. Upon selection of a Development Candidate, the JSC will meet to prepare a plan for the Development of such Development Candidate (such plan, the "Development Plan" for such Development Candidate") and to conduct a formal review of and prepare a comprehensive update to Exhibit SA-4A to the Second Amendment (the "Development Deadline Document") that reflects anticipated activities directed towards Development and commercialization of such Development Candidate through Regulatory Approval in the United States, in each case taking into consideration available information concerning such Development Candidate, the interests of SMA patients, the intellectual property and regulatory landscape and the commercial potential of the Development Candidate. The parties acknowledge and agree that Exhibit SA-4A takes into account many delays in Development and receipt of Regulatory Approval that, while possible, are not anticipated as of the Second Amendment Effective Date to be likely; Company's expectations, as of the Second Amendment Effective Date, of the activities required to obtain Regulatory Approval and its goal timelines for completing such activities are set forth in Exhibit SA-4B. When preparing the Development Plan and updating the Development Deadline Document for each Development Candidate, the JSC shall consider whether to obtain, (a) "Orphan Product" designation from the FDA, and (b) research funding from the FDA's Office of Rare Diseases or other government agencies to support human clinical trials conducted for such Development Candidate, in each case taking into consideration the protection of intellectual property rights and confidential information. The Development Plan shall set forth, in at least the level of detail included in the Company's or its Licensee's plans for developing other preclinical or clinical (whichever reflects the status of the Development Candidate at such time) pharmaceutical products, both major and minor Development activities planned to be conducted with respect to such Development Candidate by or on behalf of Company or its Affiliates or Licensees, the anticipated timeline for performing such activities, the goals of such activities and the anticipated timeline for achieving such goals. The Development Deadlines Document shall set forth the deadline by which each major Development activity must be performed by on behalf of Company or its Affiliates or Licensees if the Company wishes to avoid granting the Foundation the right to obtain a Reversionary License pursuant to Section 3.3 of the Agreement. No change can be made to any Development Plan or Development Deadline Document without the approval of the JSC unless such change is approved by the parties' respective Chief Executive Officers pursuant to Second Amendment Section 5(d) or 5(e), is implemented by Baseball Arbitration

in accordance with Second Amendment Sections 5(d) and 17, or is approved by the Foundation in accordance with Second Amendment Section 9(b)(1).

"3.2 Diligence. Prior to selection of a Development Candidate, Company shall (i) perform the activities set forth in the Research Plan in a timely and complete manner, (ii) use Commercially Reasonable Efforts to achieve the goals of the DC Research within the time and budget allotted therefor in the Research Plan, and (iii) also have the research and Development obligations set forth in Section 2.5 of the Agreement. Following selection of a Development Candidate, Company shall use Commercially Reasonable Efforts to Develop and commercialize (whether directly, through an Affiliate, or in collaboration with one or more Third Parties, through licensing or some combination of the foregoing, all in compliance with the other applicable terms of this Agreement), for the treatment, mitigation or prevention of SMA or any other disease, indication or medical condition approved in writing by Foundation, at least one Product from such Development Candidate. In the event that the Development of a Development Candidate [**] toxicology studies governed by good laboratory practices ("GLP Toxicology Studies") that causes Company to [**] that [**] is [**], then Company shall promptly notify Foundation in writing and Company shall spend up to [**] dollars ($[**]) Developing a Reversion Candidate through the start of GLP Toxicology Studies, provided that such Development does not [**] to [**] that such [**]. Upon the initiation of GLP Toxicology Studies for such Reversion Candidate, it shall be deemed a Development Candidate and Company shall have the diligence obligations set forth in the second sentence of this Section 3.2 of the Agreement. In the event that the Development of a Development Candidate [**] of [**] that [**] to [**] that such [**], then Company shall promptly notify Foundation in writing and Company shall, within [**] days of such notice, notify Foundation that Company has decided to do one of the following: (a) Develop one or more potential or actual Reversion Candidates or Lead Candidates at its own expense and in accordance with the terms and conditions of this Agreement, (b) Develop one or more potential or actual Reversion Candidates or Lead Candidates if Foundation is willing to pay for [**] percent ([**]%) of the costs of such Development for a [**] month period while the parties negotiate in good faith a separate agreement governing the further Development of such potential or actual Reversion Candidates or Lead Candidate(s); *provided*, that in the event the parties are unable to reach such separate agreement following good faith negotiations, then Company shall have [**] days following the end of such [**] month period to notify the Foundation of its decision to elect either option (a) or (c), or (c) stop all Development work on potential and actual Reversion Candidates or Lead Candidates and Development Candidates. If Company chooses option (a), then Company shall use Commercially Reasonable Efforts to Develop such Reversion Candidates and/or Lead Candidates through the start of GLP Toxicology Studies; upon the initiation of GLP Toxicology Studies for any such Reversion Candidate or Lead Candidate, it shall be deemed a Development Candidate and Company shall have the diligence obligations set forth in the second sentence of this Section 3.2 of the

Agreement. If Company chooses option (c), then upon written notice (a "Reversion Notice") to Company, Foundation shall have the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement.

"3.3 Development Benchmarks. Following designation of a Development Candidate, and in addition to Company's general diligence obligation set forth in Section 3.2 of the Agreement, Company shall use Commercially Reasonable Efforts to perform the activities set forth in the Development Plan in accordance with the timeline specified therein and to complete each activity set forth in the Development Deadline Document prior to the applicable deadline specified therein, as such Development Plan or Development Deadline Document may be amended consistent with the terms of this Agreement, with respect to Development of a Product based on such Development Candidate; provided, however, that:

"(a) the JSC shall conduct a formal review of and comprehensive update to such Development Plan and Development Deadline Document on an annual basis to reflect, on a good faith basis, information from the DC Research, ongoing clinical or supportive non-clinical trials, or other factors that may impact the activities, timelines, milestones and goals set forth such Development Plan or the deadlines set forth in such Development Deadline Document;

"(b) a failure of Company, despite Commercially Reasonable Efforts, to meet any deadline set forth in a particular Development Deadline Document (as amended by the JSC) with respect to the relevant Development Candidate (each, a "Benchmark Trigger") shall not create a breach of the Agreement, but shall instead trigger the availability of a right on the part of Foundation to obtain a Reversionary License in accordance with the following terms:

"(i) if Foundation believes a Benchmark Trigger has occurred, it shall provide written notice to Company setting forth in reasonable detail those aspects of the Development Deadline Document that have created such Benchmark Trigger.

"(ii) Company shall have [**] days to respond to a notice of Benchmark Trigger, which response shall either be (1) to cure the Benchmark Trigger (if it is capable of being cured), or (2) to propose a corrective plan to address the Benchmark Trigger, which shall take the form of a proposed amendment to the Development Deadline Document.

"(iii) if Company proposes a corrective plan to address the Benchmark Trigger, Foundation shall have [**] days to accept or reject such corrective plan. The parties may extend such [**] day period by mutual consent to engage in good faith negotiations

directed towards arriving at a mutually-agreeable form of such corrective plan with respect to such Benchmark Trigger.

"(iv) if Company fails to respond to a Benchmark Trigger notice or cure the applicable Benchmark Trigger within [**] days of such Benchmark Trigger Notice, or, following the acceptance of a corrective plan for a Benchmark Trigger by Foundation fails to use Commercially Reasonable Efforts to execute such corrective plan, or following a PTC Corporate Change the entity primarily responsible for Company's obligations under this Agreement fails to provide an M&A Certification as more fully set forth in Second Amendment Section 9(b)(4), then immediately as of such occurrence

Foundation shall have the right to obtain the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement (a "Buy-Out Right").

"(v) Foundation may exercise its Buy-Out Right by providing written notice (a "Buy-Out Notice") to Company and the first installment payment described in Section 6.1(c)(3)(iii)(A) of the Agreement, such Buy-Out Notice to be effective upon the occurrence of both (A) receipt by Company and (B) availability of funds with respect to such first installment payment (provided that such funds shall be deemed to be available on the [**] business day after the Company's receipt of such initial payment if the Company does not deposit such payment within [**] after such receipt). Upon the effectiveness of such Buy-Out Notice, the terms of Section 6.1(c)(2) of the Agreement shall apply.

"(vi) notwithstanding the foregoing, if Foundation fails to exercise a Buy-Out Right within [**] years of the date of the accrual of such Buy-Out Right, and other than with respect to the circumstances giving rise to such Buy-Out Right Company is in compliance with the terms of this Agreement, then such Buy-Out Right shall lapse and no longer be exercisable by Foundation. For clarity, the foregoing operates on a Buy-Out Right by Buy-Out Right basis, with Foundation having a full [**] year period to exercise each Buy-Out Right.

"3.4 Decisions to Discontinue Development or Commercialization.

(a) At the request of Company, the JSC shall determine, based on a comparison of test data for a particular Development Candidate (both alone and in combination with another treatment) and for the applicable Available Product and through the application of objective criteria previously established by the JSC (or by a mutually agreed independent technical expert if the JSC is not able to agree upon such objective criteria within [**] days after either party provides written notice to the other that

it intends to arrange for a technical expert to decide such criteria), which criteria shall include without limitation [**] and [**], whether such Development Candidate (both alone and in combination with another treatment) appears to be less desirable as a therapeutic option in the Field than such Available Product. If, following such a determination, Company informs Foundation in writing that it intends to cease further Development and commercialization of the applicable Development Candidate, then Foundation shall have: (i) upon Foundation's written notice to Company (such notice, a "Reversion Notice"), a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement if such Available Product [**] (a "[**]"), or (ii) a Buy-Out Right if such Available Product is a [**], such right to be exercisable by Foundation on the terms provided in Sections 3.3(b)(v) and (vi) of the Agreement.

(b) Company shall notify Foundation in writing if Company has, in its good faith judgment, decided that a particular Development Candidate is not commercially viable, which decision shall not be based, in whole or in part, upon the size of the addressable patient population for such Development Candidate. Such notice shall include a written explanation of the basis for Company's decision. Unless the parties enter into a separate agreement pursuant to which [**] the Development or commercialization of such Development Candidate, Foundation shall, upon written notice to Company (such notice, a "Reversion Notice"), have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement.

(c) Company shall notify Foundation in writing if Company has, in its good faith judgment, determined that (i) based on advice of outside patent counsel, the pharmaceutical preparation, composition of matter, method of manufacture or method of use of a particular Development Candidate is covered by at least one issued and apparently valid and enforceable United States Patent of a Third Party and (ii) it is not possible for Company (or its Affiliate or Licensee, as applicable) to obtain a license under such Third Party Patents on commercially reasonable terms (a "Third Party Patent License"). Upon receipt of such notice from Company and provision by Foundation of a written notice to Company (such notice, a "Reversion Notice"), Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement.

(d) Company shall notify Foundation in writing if Company has, in its good faith judgment, decided to cease all Development and commercialization of a particular Development Candidate and it believes that such cessation is not a breach of the obligations set forth in Section 3.2 of the Agreement. Such notice shall include a written explanation of the basis for Company's belief. Unless the Foundation notifies Company in writing that it does not agree with such belief and that Company is

obligated to continue Development and commercialization of such Development Candidate in accordance with Sections 3.2 and 3.3 of the Agreement, then upon Foundation's written notice to Company (such notice, a "Reversion Notice"), Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement."

(b) The parties acknowledge their continued interest in Research Project B in the area of [**] which is [**], and their good faith intention to continue negotiations (including negotiations with any Third Parties) with respect to finding a way to fund and advance research directed towards Research Project B. Therefore, notwithstanding anything to the contrary in this Second Amendment, the amendments effectuated by this Second Amendment shall not apply to such Research Project B or any Lead Candidates identified during the course of such Research Project B. Instead, the terms of the Agreement (including those amendments implemented pursuant to the First Amendment) as they existed prior to amendment by this Second Amendment shall continue to apply, after the Second Amendment Effective Date, exclusively to such Research Project B and any Lead Candidates identified during the course of such Research Project B.

9. PTC Corporate Change:

(a) M&A Approval Request. Company shall have the option to notify a designated representative of Foundation in writing (an "M&A Approval Request") no later than [**] days prior to the entry into a definitive written agreement involving a PTC Corporate Change. Such M&A Approval Request shall include the identity of the proposed acquiring or merging entity or entities, the expected relationship (if any) between Company and its Affiliates, Company's shareholders, and Company's management following such PTC Corporate Change, and the expected impact of such PTC Corporate Change on Company's obligations under the Agreement. In addition, subject to appropriate confidentiality protections and the consent of the potential acquiring or merging entity or entities (to the extent such information is information of the potential acquiring or merging entity or entities or relates to the economics or financial terms of the

potential PTC Corporate Change), Company shall promptly provide to Foundation's designated representative any supplemental information concerning such potential PTC Corporate Change as Foundation shall reasonably request. If Foundation responds to such M&A Approval Request by consenting to such proposed PTC Corporate Change prior to Company's entry into a definitive written agreement involving a PTC Corporate Change, then the terms and conditions of this Agreement shall remain in full force and effect without alteration, and Foundation shall sign such documents and provide such consents as may be reasonably required to effectuate the proposed PTC Corporate Change as described in such M&A Approval Request. If Foundation does not respond prior to Company's entry into a definitive written agreement involving a PTC Corporate Change, or responds by denying consent prior to Company's entry into a definitive written agreement for such PTC Corporate Change, then the consequences in Second Amendment Section 9(b) shall apply. Having given an M&A Approval Request to Foundation, Company shall not enter into a definitive written agreement involving a PTC Corporate Change contemplated in such M&A Approval Request prior to the earlier of (i) receipt

of a response from Foundation as specified in this Second Amendment Section 9(a), or (ii) [**] days following the provision of such M&A Approval Request to Foundation.

(b) Parallel Notification Option. If with respect to a particular definitive written agreement that would result in the a PTC Corporate Change, Company has not given the M&A Approval Request provided for in Second Amendment Section 9(a), or if Company has given such M&A Approval Request and Foundation has either failed to respond within [**] days or responded by denying consent, then within [**] of entering into such definitive written agreement, Company shall provide written notice thereof to a designated representative of Foundation (the "M&A Notice"). Such M&A Notice shall include the identity of the proposed acquiring or merging entity or entities, the expected relationship (if any) between Company and its Affiliates, Company's shareholders, and Company's management following such PTC Corporate Change, and the expected impact of such PTC Corporate Change on Company's obligations under the Agreement. Unless otherwise agreed by Foundation, (i) in connection with an M&A Notice given by Company or (ii) if Foundation does not respond to, or denies, an M&A Approval Request pursuant to Second Amendment Section 9(a), then the following terms and conditions shall apply effective upon Company's entry into such definitive written agreement:

(1) Notwithstanding the provisions of Second Amendment Sections 5(b)(x), 5(c)(ii), 5(d) and 5(e), any updates or amendments to the Development Plan (including the initial preparation thereof; provided, however, if as of Company's entry into such definitive written agreement a DC has been selected, but no Development Plan exists, any failure of the JSC to agree on preparation of an initial Development Plan shall be escalated to the Chief Executive Officers and, if required, referred to Baseball Arbitration as provided in Second Amendment Section 5(d)) or Development Deadline Document shall require the prior approval of Foundation in its sole discretion.

(2) Notwithstanding the provisions of Second Amendment Section 5(e), if following the achievement of Proof-of-Concept the JSC is unable to reach a unanimous decision on any matter within its jurisdiction (other than an update or amendment to the Development Plan or Development Deadline Document), and the parties' respective Chief Executive Officers are not able to resolve the matter in good faith as set forth in Second Amendment Section 5(e), then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment;

(3) If this Agreement is not assigned upon the consummation of such PTC Corporate Change to the entity that gained control of Company or its assets as a result of such PTC Corporate Change, then such entity shall enter into a written agreement with Foundation wherein such entity shall guarantee the performance of Company's obligations pursuant to this Agreement; and

(4) The entity that, following the PTC Corporate Change, will be principally responsible for the obligations of Company under the Agreement shall have [**] days following the consummation of such PTC Corporate Change to (A) have a member of the executive management team of such entity who is responsible for Development of products being developed by Company prior to the PTC Corporate Change participate in a meeting with representatives of Foundation at the Foundation's headquarters to discuss

such entity's plans for conducting the DC Research (if not completed prior to the PTC Corporate Change) and for Developing Products based on Development Candidates and (B) provide to Foundation a written certification (the "M&A Certification") by an authorized officer of such entity (i) affirming such entity's intention to perform the Company's obligations under the Agreement, (ii) summarizing in reasonable detail such entity's plans with respect to conduct of any part of the DC Research not performed by Company as of the effectiveness of the PTC Corporate Change, including a demonstration that sufficient funds, FTEs and other resources have been allocated to the performance of such DC Research, and (iii) summarizing in reasonable detail such entity's plans with respect to execution of the Development Plan and completion of the activities set forth in the Development Deadline Document prior to the deadlines specified therein, including a demonstration that sufficient funds, FTEs and other resources have been allocated to the performance of such Development, and such entity's business plans for the Development Candidates; *provided*, that failure to provide such M&A Certification in the time frame specified in this Second Amendment Section 9(b)(4) shall entitle Foundation to exercise the Buy-Out Right specified in Section 3.3(b)(iv) of the Agreement, such right to be exercisable by Foundation on the terms provided in Sections 3.3(b)(v) and (vi) of the Agreement.

(c) Special Provisions in Connection with M&A Approval Request and/or M&A Notice. The parties recognize the special sensitivity of the information contained in an M&A Approval Request and/or and M&A Notice, and agree that any such notice and its contents are Confidential Information of Company pursuant to this Agreement. In addition, Foundation agrees not to use, and to use Commercially Reasonable Efforts to prevent use by any of its Affiliates, of any material non-public information contained in an M&A Approval Request and/or and M&A Notice for the purposes of transactions involving the equity or debt securities of either (i) Company or its Affiliates or (ii) any entity participating in the potential or actual transactions resulting in the PTC Corporate Change described in such M&A Approval Request and/or and M&A Notice. In addition, following receipt of an M&A Approval Request and/or and M&A Notice that includes material non-public information given while Company is subject to the periodic reporting requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, and for so long as the material information included in the M&A Approval Request and/or M&A Notice is non-public, unless specifically invited in writing by Company to do so, Foundation shall not, and shall use Commercially Reasonable Efforts to prevent its Affiliates from, in any manner, directly or indirectly, (a) publicly effecting or seeking, initiating, offering or proposing to effect or cause or participating in (whether publicly or otherwise and whether directly or through a Third Party), any tender or exchange offer, merger, consolidation or other business combination involving Company; or any "solicitation" of "proxies" (as such terms are used in the proxy rules of the United States Securities and Exchange Commission) or consents to vote any voting securities of Company; (b) forming, joining or in any way participating in a "group" (as defined under the Securities Exchange Act of 1934, as amended) with respect to any voting securities of Company (or security convertible into rights to acquire any voting security of Company); (c) taking any action which could

reasonably be expected to force Company to make a public announcement regarding any of the types of matters set forth in (a) above; or (d) entering into any agreements, discussions or arrangements with any Third Party with respect to any of the foregoing. In addition, Foundation shall not, and shall use

Commercially Reasonable Efforts to prevent its Affiliates from, causing or knowingly permitting any of its or their respective directors, officers, employees, investment bankers (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), attorneys (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), accountants (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), or other advisors or representatives (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable) to initiate or participate in any of the actions described in the foregoing clauses (a), (b), (c), or (d).

10. Partnering.

(a) Company shall have the primary responsibility to evaluate the need for and timing of Collaboration Activities.

(b) During any period in which Company is not actively pursuing Collaboration Activities, Company shall report to the JSC on [**] basis Company's views of the partnering/collaboration marketplace for drug discovery and lead optimization efforts at a similar stage to efforts under the DC Research or, if a Development Candidate has been declared, the partnering/collaboration marketplace for development candidates at a similar stage of development to such Development Candidate.

(c) If Company determines to actively pursue Collaboration Activities, whether at its own initiative or in response to inquiries from Third Parties, Company will first seek input from the JSC on the nature, scope, and potential terms of a transaction arising in connection with such Collaboration Activities, as well as a rank-ordered summary list of preferred potential counterparties to such transaction. To the extent prepared by Company rather than received by Company from a potential counterparty, Company shall also provide the JSC with an opportunity to review a draft term sheet and related materials in support of its proposed Collaboration Activities. The JSC shall promptly provide input on Company's overall approach to Collaboration Activities, as well as specific input on any term sheet or related materials provided to the JSC.

(d) Prior to Company commencing formal term sheet negotiations or contractual negotiations with any Third Party in connection with Collaboration Activities, Company shall first notify Foundation in writing concerning such negotiations (a "Partnering Notice"). Such Partnering Notice shall be accompanied by any available drafts of term sheets or contracts, or if not available, a summary of the proposed transaction to the extent available, in either case subject to redactions of financial terms to the extent required to comply with any confidentiality agreements with the potential counterparty. Following receipt of a Partnering Notice, Foundation and Company shall have the following rights and obligations in connection with the proposed transaction described in the Partnering Notice (regardless of whether, in the course of negotiations, the terms change from those described the original Partnering Notice):

(i) Foundation shall designate a representative to serve in an advisory capacity with respect such transaction and, if requested by Company, to participate in negotiations subject to appropriate confidentiality protections; *provided, however,* that such representative shall not have the power to commit Foundation to enter into any

amendments to the Agreement absent formal written approval by an appropriately authorized officer of Foundation.

(ii) Foundation may designate legal counsel and [**] of Foundation, each subject to appropriate confidentiality protections, to review and provide comments upon proposed term sheets and contracts for such transaction subject to reasonable time frames consistent with overall progress and status of negotiations and not less than [**]percent ([**]%) of the timeframe specified by Company for the receipt of comments from its senior management. For clarity, such reasonable time frames may be as short as [**] if, in Company's reasonable judgment, such time frames are required to support a successful negotiation process and such time frames are not less than [**] percent ([**]%) of the timeframe specified by Company for the receipt of comments from its senior management. If requested by the potential counterparty, Company shall have the right to redact financial terms from such term sheets and contracts. In reviewing and commenting on such proposed term sheet and contracts, Foundation counsel and designated representative shall indicate the relative importance of their comments, and if practicable a range of potential responses for negotiation purposes. Company shall use Commercially Reasonable Efforts to implement comments and negotiating positions suggested by Foundation's counsel and/or representative, with due consideration given to the relative importance assigned to the comments and reflecting the outcome of any discussions between Company and Foundation's counsel and/or representative with respect to modification of such comments. In addition, Company shall, as non-negotiable contractual terms, require (1) that the counterparty commit, in response to the JSC's invitation, to sending a representative of such counterparty to such JSC meetings or portions of meetings as the JSC shall request for the purposes of informing, discussing and serving in an advisory role with respect to decisions regarding the progress of and all future plans for the DC Research and the Development and commercialization of the Reversion Candidates, Development Candidate(s) and Product(s) that are the subject of the agreement between Company and such counterparty, (2) an acknowledgement that the JSC shall remain the sole governance body for all research, Development and commercialization decisions regarding the DC Research and the Development and commercialization of Reversion Candidates, Development Candidates and Products that are the subject of the agreement between Company and such counterparty (which acknowledgement may include a clarification that the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision), (3) that to the extent the counterparty will assume responsibility for Development of a Development Candidate or Product in any country, that such counterparty assume the obligations and rights of Company pursuant to Second Amendment Section 13 in that country, (4) an acknowledgement that the counterparty's rights and licenses from Company with respect to Reversion Candidates, Development Candidates and Products will terminate upon a Special Termination or Company's receipt of a Reversion Notice or Buy-Out Notice and an obligation in such circumstance for the

counterparty to grant the licenses and rights specified in Section 3 of this Second Amendment and Section 6.1(c)(2) of this Agreement (including licenses and rights to (A) all intellectual property that, if developed, acquired or otherwise Controlled by Company, rather than such counterparty, would be

Company Technology or Data ("Licensee Technology" and "Licensee Data", respectively) and (B) all INDs, NDAs or similar regulatory filings made or obtained by such counterparty with respect to the relevant Reversion Candidates, Development Candidates and Products) and perform the activities specified therein in each case as if such counterparty were Company, and (5) third party beneficiary rights for Foundation in the event that such counterparty fails to fulfill any of the foregoing obligations.

(iii) Prior to the conclusion of contractual negotiations pursuant to such Partnering Notice, Company shall schedule at least [**] with representatives of the negotiating team of potential counterparties to the transaction and the representatives designated by Foundation pursuant to the foregoing subsections (i) and (ii) to discuss Foundation's goals and interests with respect to such proposed transaction. Unless otherwise agreed by Foundation, Company shall use Commercially Reasonable Efforts to cause [**] to take place in person at a location convenient to the New York metropolitan area.

(e) Prior to Company entering into a definitive written agreement with any Third Party in connection with Collaboration Activities, Company shall seek the review and approval of the JSC by providing the members of the JSC a proposed final draft of the definitive written agreement and a summary [**] of the proposed transaction, including an overview of any items or terms subject to finalization in the draft provided. If required by the Company's confidentiality agreement with the potential counterparty, Company shall have the right to redact financial terms from such proposed final draft of the definitive written agreement. As promptly as reasonably possible, but in no event later then [**] business days following receipt by the JSC members of such proposed final draft of the definitive written agreement and summary, the JSC shall convene a meeting to either approve or deny for such proposed transaction; *provided, however*, that if Company has otherwise complied with requirements of this Second Amendment Section 10, Foundation shall only be entitled to cast its JSC vote against such proposed transaction if it agrees either (i) to fund [**] percent ([**]%) of ongoing Development and commercialization costs for the applicable Development Candidate(s) or Product(s), or (ii) [**] and any related rights pursuant to Section [**]; and *provided further*, that failure of either party to make itself available within the time frames specified in this Second Amendment Section 10(e) shall entitle the other party to either approve or deny the proposed transaction in the name of the JSC without the requirement of holding an actual JSC meeting. If the JSC denies approval in accordance with this Second Amendment Section 10(e), Company shall not enter into such proposed definitive written agreement, but shall have the right to continue the applicable negotiations consistent with this Second Amendment Section 10 for the purposes of achieving a form of such definitive written agreement acceptable to the JSC.

(f) Following the entry into a transaction pursuant to this Second Amendment Section 10, the following additional terms and conditions will apply:

(i) The JSC shall continue as the sole governance body for the conduct of the DC Research and the Development and commercialization of Reversion Candidates, Development Candidates and Products and shall continue to have all the rights and responsibilities specified in Second Amendment Section 5.

(ii) The JSC shall invite the representative of the counterparty designated pursuant to Second Amendment Section 10(d)(ii)(1) to such JSC meetings, or portions of meetings, as the JSC shall deem advisable for the purposes of informing, discussing and serving in an advisory role with respect to decisions regarding the progress of and all future plans for the DC Research and the Development and commercialization of the Reversion Candidates, Development Candidate(s) and Product(s) that are the subject of the agreement between Company and such counterparty.

11. Company Payments to Foundation and Related Provisions: Sections 4.3, 4.4, 4.5, 4.6 and 4.7 of the Agreement (captioned "Milestone Donation by Company", "Reporting of Product Revenues", "Exchange Rate; Manner and Place of Payment", "Taxes" and "Audits", respectively) shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"4.3 Payments by Company. Company will make the following payments to Foundation in connection with Product Revenues:

"(a) Company will make payments as specified below to Foundation up to a maximum amount equal to [**] by [**] pursuant to the Agreement (the "SMAF Funding Amount," which, for clarity, includes [**] pursuant to the [**] defined below (such total amount, the "Repayment Amount"). For the purposes of this Section 4.3 of the Agreement, the "[**]" shall be [**] unless and until both (i) a Product has achieved Worldwide Net Sales of at least [**] US dollars ($[**]) in any calendar year; and (ii) the SMAF Funding Amount received with respect to such Product equals or exceeds a total of [**] US dollars ($[**]), upon the occurrence of which the [**].

"(b) In the event that Company and/or its Affiliates sells Products, then Company shall pay the Repayment Amount to the Foundation by making installment payments to the Foundation, each of which shall be equal to [**] percent ([**]%) of Net Sales received by Company and its Affiliates in the applicable calendar quarter and each of which shall be paid in U.S. dollars, by wire transfer to an account specified by the Foundation, within [**] days of end of such calendar quarter. The first such installment payment shall be paid for the first calendar quarter following the first calendar year in which Net Sales equaled or exceeded [**] U.S. Dollars (US$[**]) (the "Sales Threshold"). An additional installment payment shall be paid to the Foundation for each subsequent calendar quarter until such time as the sum of all installment payments made pursuant to this Section 4.3(b) of the Agreement, together with all installment payments made pursuant to Section 4.3(c) of the Agreement, equals the Repayment Amount. If the [**], Company shall make additional payments until the updated Repayment Amount has been met.

"(c) In the event that Company and/or its Affiliates enters into one or more license agreements for the development, manufacture, use, distribution, promotion or sale of a Drug Candidate or Product in one or more territories, then Company shall repay the Repayment Amount by making installment payments to the Foundation, each of which shall be equal to [**] percent ([**]%) of PTC License Income received by Company and its Affiliates in the applicable calendar quarter and each of which shall be paid in U.S. dollars, by wire transfer to an account specified by the Foundation, within [**] days of the end of such calendar quarter. The first quarter during which such installment payments shall be paid shall be the first calendar quarter following the first calendar year in which both of the following criteria are met: (i) a Licensee makes or has previously made its First Commercial Sale and (ii) Worldwide Net Sales equal or exceeded [**] U.S. Dollars (US$[**]). An additional installment payment shall be paid to the Foundation for each subsequent calendar quarter until such time as the sum of all installment payments made pursuant to this Section 4.3(c) of the Agreement, together with all installment payments made pursuant to Section 4.3(b) of the Agreement, equals the Repayment Amount. If the [**], Company shall make additional payments until the updated Repayment Amount has been met. Notwithstanding the foregoing, if the payments owed pursuant to this Section 4.3(c) of the Agreement would, when combined with other payments owed by Company to Third Parties in connection with the receipt of such PTC License Income, exceed [**] percent ([**]%) of such PTC License Income, then the payments owed pursuant to this Section 4.3(c) of the Agreement and such other payments owed by the Company to Third Parties shall all be automatically reduced pro rata until the combined payments no longer exceed [**] percent ([**]%) of such PTC License Income; provided, however, that this reduction shall only be available with respect to payments under this

Section 4.3(c) of the Agreement if all other payments owed by Company to Third Parties in connection with the receipt of such PTC License Income are also subject to such pro rata reduction.

"4.4 Reporting of Net Sales and PTC License Income. From and after such time as Company first receives any Net Sales or PTC License Income and until such time as Company has paid in full the amount due under Section 4.3 of the Agreement (if any), Company shall deliver to the Foundation (or a Third Party designated in writing by the Foundation) quarterly written reports of Net Sales and PTC License Income received by Company and its Affiliates, which reports shall (a) separately indicate the total Net Sales and PTC License Income received, (b) show how Net Sales were calculated from the gross amounts received by Company and its Affiliates, with each deduction from gross amounts being separately itemized, (c) show how PTC License Income was calculated, and (d) itemize any amounts received by Company and its Affiliates from a Licensee that were excluded from PTC License Income and the rationale for such exclusion. Company shall keep, and shall cause its Affiliates to keep, complete and accurate records pertaining to the receipt of Net Sales and PTC License Income in sufficient detail to permit the Foundation to confirm the accuracy of such reports.

"4.5 Exchange Rate; Manner and Place of Payment. All payments hereunder shall be payable in U.S. dollars; provided, that in the event that, by reason of applicable legal requirement in any country, it becomes impossible or illegal for a payor to transfer,

or have transferred on their behalf, royalties or other payments to the payee, the payor shall promptly notify the payee of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of the payee in a recognized banking institution designated by the payee or, if none is designated by the payee within a period of [**] days, in a recognized banking institution selected by the payor and identified in a notice given to the payee. When conversion of payments from any foreign currency is required for purposes of a calculation under this Agreement that relates to a payment from one party to the other, such conversion shall be at the exchange rate used by the payor (or, where applicable, a Licensee or licensee of Foundation) throughout its accounting system (which shall, in any event, be commercially reasonable) during the quarter for which such report is due. All payments owed under this Agreement shall be made by check, or by wire transfer in immediately available funds to a bank and account designated in writing by the party entitled to receive payment, unless otherwise specified in writing by such party.

"4.6 Taxes. Each party will pay any and all taxes levied on account of any payments made to it under this Agreement out of the amounts it is to receive hereunder. If any taxes are required to be withheld by the party making payment, such party will (a) deduct such taxes from the payment made by it, (b) timely pay the taxes to the proper taxing authority, (c) send proof of payment to the other party and certify its receipt by the taxing authority within [**] days following such payment, and (d) be deemed to have paid such amount to the other party hereunder.

"4.7 Audits. The Foundation shall have the right to cause an independent, certified public accountant reasonably acceptable to Company to audit the records of Company and its Affiliates to confirm the accuracy of (a) Company's reports of Net Sales and PTC License Income, (b) Company's accounting pursuant to Second Amendment Section 2(d) or 4(a) of its use of internal resources and the out-of-pocket expenses that Company incurred in accordance with the Research Plan, (c) the amount specified in Second Amendment Section 2(d) as the amount spent by Company on DC Research between [**] and [**], and (d) Company's invoice pursuant to Second Amendment Section 2(d) with respect to the amounts it spent between [**] and the Second Amendment Effective Date, in each case for a period covering not more than the preceding [**] years. Such audits may be exercised during normal business hours upon reasonable prior written notice to Company and no more than [**] per year. If an audit reveals that Company has underpaid any amount due to the Foundation, overcharged Foundation pursuant to Second Amendment Section 2(d) or overstated in Second Amendment Section 2(d) the amount that it spent on DC Research between [**] and [**], Company shall pay all such amounts to the Foundation within thirty (30) days of receiving the Foundation's audit report. The Foundation shall bear the full cost of such audit unless such audit discloses (i) an underreporting of Net Sales or PTC License Income by Company of more than [**]% during any calendar year, (ii) an over-reporting of internal resources and the out-of-pocket expenses of more than [**]% during any calendar year or (iii) that Second Amendment Section 2(d) over-states by more than [**]% the amount that Company spent on DC Research between [**] and [**], in which case, Company shall bear the full cost of such audit."

12. Reversionary License. Section 6.1(c) of the Agreement (captioned "Reversionary Licenses to Data and Company Technology") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"(c) **Reversionary Licenses to Data and Company Technology.**

"(1) In the event that:

"(i) During the term of the DC Research, Company fails to perform its obligations as set forth in Second Amendment Section 2 with respect to conduct of the DC Research, and does not remedy such failure to comply within [**] days after notice thereof from Foundation; *provided, however,* that in the event [**], the parties shall promptly meet to negotiate in good faith the [**], and Company's right to cure any failure under this Section 6.1(c)(1)(i) shall be extended to the longer of (xx) [**] days after Foundation provides Company written notice that it wishes to terminate such good faith negotiations, or (yy) such other period as the parties may agree in connection with a mutually-agreed plan to address [**];

"(ii) Company is otherwise in material breach of this Agreement with respect to the DC Research and does not remedy such breach within [**] days after notice of such breach from Foundation; or

"(iii) Company is in material breach of its obligations set forth in Section 3.2 of the Agreement, and does not remedy such breach within [**] days after notice thereof from Foundation;

"then, in any such case, Foundation shall have the option to declare the effectiveness of the terms and conditions specified in Section 6.1(c)(2) of the Agreement, such option to be exercised by providing written notice to Company (a "Reversion Notice") within the [**] period following the last date on which Company could have cured such failure or breach pursuant to this Section 6.1(c)(1) of the Agreement.

"(2) Effective upon receipt of a Reversion Notice pursuant to Section 3.2, 3.4(a)(i), 3.4(b), 3.4(c) or 3.4(d) of the Agreement or within the time period specified in Second Amendment Section 3(d) or Section 6.1(c)(1) of the Agreement, or upon the effectiveness of a Buy-Out Notice pursuant

to Section 3.3(b)(v) of the Agreement (regardless whether the Foundation obtained its Buy-Out Right pursuant to Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement or Second Amendment Section 9(b)(4)), the following terms and conditions shall apply:

"(i) Company shall, and it hereby does, grant to Foundation an exclusive worldwide license, including the right to grant sublicenses, under any Company Technology, Licensee Technology, Data or Licensee Data that relates to a pharmaceutical preparation, composition of matter, method of manufacture and/or method of use in the Field, of Reversion Candidates and/or Products containing one or more Reversion Candidates, solely for the purpose of

researching, developing, making, having made, using, selling, having sold, offering for sale and importing Reversion Candidates and Products containing Reversion Candidates (such Products, "Reversion Products") in the Field (such license being referred to herein as the "Reversionary License"). The Reversionary License shall be fully-paid up and royalty free unless the Foundation obtains the Reversionary License pursuant to (A) Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement, or Second Amendment Section 9(b)(4), in which case the licensing fees, royalties and other terms set forth in Section 6.1(c)(3) of the Agreement shall also apply or (B) Second Amendment Section 3(d) of the Agreement, in which case the royalties, [**], and other terms set forth in Section 6.1(c)(4) of the Agreement shall also apply;

"(ii) Company shall, and it hereby does, grant to Foundation a fully-paid up, royalty-free, non-exclusive, and worldwide license, including the right to grant sublicenses, to (xx) Company Technology and Licensee Technology to the extent not exclusively licensed pursuant to Section 6.1(c)(2)(i) of the Agreement, (yy) Data and Licensee Data, and (zz) Company Base IP, in each case solely to the extent (1) reasonably necessary for Foundation to exercise its rights under the Reversionary License or (2) useful for Foundation to exercise its rights under the Reversionary License and used or contemplated to be used in the DC Research or pursuant to the Development Plan (as applicable); *provided*, that the license granted to Foundation in this Section 6.1(c)(2)(ii) of the Agreement [**] or [**] or [**];

"(iii) Company shall reasonably cooperate with Foundation in order to enable Foundation to continue, initiate or re-initiate the Development, manufacture and commercialization of the Reversion Candidates or Reversion Products, such cooperation and assistance to be provided in a timely manner (having regard to the nature of the cooperation or assistance requested) and including without limitation (in each case with respect to the Reversion Candidates or Reversion Products): (A) within [**] months of the Reversion Notice or Buy-Out Notice: (1) transferring or granting a right of reference to any INDs, NDAs, or similar regulatory filings made or obtained by Company or its Affiliate or Licensee; (2) providing a copy of all Data and Licensee Data and all reports and other information that were (or should have been) accessible to Foundation via the shared electronic collaboration space described in Second Amendment Section 4(a); and (3) providing reasonable quantities of existing stock of materials (other than (1) materials [**] or (2) materials [**] in Company's possession or under its control and (xx) that are specific to, or were used or were contemplated to be used in, the DC Research or the Development Plan, and are not commercially available from Third Parties, (yy) that are reasonably necessary or useful for continued research, Development or commercialization of Reversion Candidates in the Field, and (zz) the transfer of which would not [**] trigger any contractual obligation to make payments to a Third Party (other than a Licensee); provided, however, that any subsequent transfers of such materials by Foundation to Third Parties shall be subject to the

terms of a materials transfer agreement reasonably acceptable to Company; (B) permitting Foundation to purchase, for a period of up to [**] years (or less if Foundation obtains an alternative validated, supply source within such [**] year period), Reversion Candidates and Reversion Products [**], but only to the extent (1) such Reversion Candidates or Reversion Products are manufactured by Company itself (as opposed to under a Third Party manufacturing contract) or (2) such Reversion Candidates or Reversion Products are manufactured for the Company by a Third Party and the agreement pursuant to which such Reversion Candidates or Reversion Products are manufactured (xx) provides for manufacture of other active pharmaceutical ingredients or pharmaceutical products that are not Reversion Candidates or Reversion Products, (yy) is not assignable to Foundation or (zz) has not been assigned to Foundation; (C) permitting Foundation to purchase [**] all or any part of Company's worldwide unsold inventory of such Development Candidate or Product together with any raw materials and work-in-process relating to such Development Candidate or Product; (D) upon Foundation's request, using Commercially Reasonable Efforts to assign to Foundation any Third Party manufacturing contracts relating to Reversion Candidate or Reversion Product; (E) upon Foundation's request, using Commercially Reasonable Efforts to assign to Foundation any Third Party license agreements relating to such Reversion Candidate or Reversion Product; and (F) providing prompt technical assistance as requested by Foundation [**] for [**] months after the Reversion Notice or Buy-Out Notice;

"(iv) Foundation, at its own expense, shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with respect to losses arising out of or related to its activities pursuant to the Reversionary License and other rights granted in this Section 6.1(c)(2) of the Agreement, and Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request;

"(v) At Foundation's option, on a license-by-license basis, either (i) Foundation may request in writing that Company use Commercially Reasonable Efforts to secure the assignment to Foundation or its designee any licenses granted by Company to Licensees; or (B) upon written notice from Foundation all licenses granted by Company to Licensees shall automatically terminate and Licensees shall be obligated to perform the obligations set forth in this Section 6.1(c)(2) of the Agreement as if they were Company. Company shall include in each agreement with a Licensee an acknowledgement by Licensee of the foregoing and a provision that grants Foundation third party beneficiary status with respect to Licensee's performance (or failure to perform) such obligations;

"(vi) The rights and obligations of the parties pursuant to Article 2 of the Agreement, Article 3 of the Agreement, Sections 4.3, 4.4, 4.5, 4.6, 4.7(a) and 6.3 of the Agreement, First Amendment Sections 5, 6 and 7, and Second Amendment Sections 2 (including Foundation's obligations to fund the DC Research), 3, 4

(except for the final report described in Second Amendment Section 4(b)(ii)), 5, 6, 7, 9, 10, 13, 17, 18(a), 18(b), 18(c), 18(d)and 18(f) shall also terminate; and

"(vii) Notwithstanding the foregoing provisions of Section 6.1(c)(2) of the Agreement, in no event shall Company be required to take any actions pursuant to Section 6.1(c)(2) of the Agreement that, in the good faith judgment of outside counsel to the Company, would infringe any Third Party intellectual property rights (and no non-infringing alternative is identified after a reasonable inquiry) or trigger a breach of any contractual obligations of Company with respect to a Third Party (other than a Licensee)."

"(3) In addition to the provisions of Section 6.1(c)(2) of the Agreement, if Foundation obtains the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement pursuant to Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement, or Second Amendment Section 9(b)(4), then (A) Foundation shall [**] and (B) Foundation shall make the following payments to Company with respect to such Reversionary License and rights:

"(i) If [**] pursuant to this Agreement (which, for clarity, [**] as of the accrual of the applicable Buy-Out Right, then Foundation shall pay to Company a licensing fee equal to [**] U.S. dollars ($[**]) as specified in (iii) below.

"(ii) In the alternative, if [**] with respect to [**] pursuant to this Agreement (which, for clarity, [**]) as of the accrual of the applicable Buy-Out Right, then Foundation shall pay, as specified in (iii) below, to Company a licensing fee that is equal to the sum of [**] U.S. dollars ($[**]) plus *x*, where *x* equals the lesser of (A) [**] and (B) [**] U.S. dollars ($[**]). For clarity, such licensing fee shall never exceed [**] U.S. dollars ($[**]).

"(iii) Foundation shall pay the licensing fee set forth in (i) or (ii) above in three installments: (A) a first installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation simultaneously with Foundation's notice that it is exercising such Buy-Out Right; (B) a second installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation by the [**]month anniversary of Foundation's notice that it is exercising such Buy-Out Right, provided that Company has complied with its obligations pursuant to Section 6.1(c)(2) of the Agreement in good faith and responded promptly and adequately to any Foundation notices detailing any alleged lack of such good faith compliance; and (C) a final installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation (x) by the [**]month anniversary of Foundation's notice that it is exercising such Buy-Out Right, provided that Company has complied with its obligations pursuant to Section 6.1(c)(2) of the Agreement in good faith and responded promptly and adequately to any Foundation notices detailing any alleged lack of such good faith compliance, or (y) if earlier, the date upon which Foundation is satisfied that Company has fully performed all obligations of Company set forth in Section 6.1(c)(2) of the Agreement;

"(iv) [**], Foundation shall pay to Company the following percentage royalties (on a Reversion Product-by-Reversion Product basis) on product revenues (based on the definition of Product Revenues in the Agreement, but substituting "Reversion Product" for "Product" and "Foundation" for "Company" as the context requires) of any Reversion Product, such payments to be made on a quarterly basis in arrears no later than [**] days following the end of the applicable quarter:

Stage of Reversion Candidate Upon Exercise of Buy-Out Right by Foundation	**Royalty on Product Revenues of applicable Reversion Product**
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

"For the purposes of the foregoing table, [**] shall mean [**]. The royalties in the foregoing table shall commence, on a country-by-country basis, upon the first commercial sale (based on the definition of First Commercial Sale in the Agreement, but substituting "Foundation" for "Company" as the context requires) of the applicable Reversion Product in such country, and continue for the longer of (xx) [**] from the date of such first commercial sale or (yy) the date of expiration of the last Company Patent covering the applicable Reversion Product within the applicable country. Foundation shall comply with the applicable provisions of Sections 4.4, 4.5, 4.6 and 4.7 of the Agreement with respect to such royalty payments, substituting "Company" for "Foundation" and vice versa as the context may require."

"(4) In addition to the provisions of Section 6.2(c)(2) of the Agreement, if Foundation obtains the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), then Foundation shall make the following payments to Company with respect to the Reversionary License and shall have the following obligations to Company:

"(i) Foundation shall (A) [**] and (B) make royalty payments to Company, on a Reversion Product-by-Reversion Product basis, equal to [**] percent ([**]%) of product revenues (based on the definition of Product Revenues in the Agreement, but substituting Reversion Product for Product and Foundation for Company as the context requires) of any Reversion Product, such payments to

be made on a quarterly basis in arrears no later than [**] days following the end of the applicable quarter. Such royalty payments shall commence, on a country-by-country basis, upon the first commercial sale (based on the definition of First Commercial Sale in the Agreement, but substituting "Foundation" for "Company" as the context requires) of the applicable Reversion Product in such country, and continue for the longer of (xx) [**] from the date of such first commercial sale or (yy) the date of expiration of the last Company Patent covering the applicable Reversion Product within the applicable country. Foundation shall comply with the applicable provisions of Sections 4.4, 4.5, 4.6 and 4.7 of the Agreement with respect to such royalty payments, substituting "Company" for "Foundation" and vice versa as the context may require."

"(ii) Before granting any Third Party an exclusive sublicense of the Reversionary License for any purpose that includes commercializing any Reversion Product in the United States, [**], for a period of up to [**] months, [**] would be [**] and [**] to [**]. If the [**], by the end of such [**] month period, a [**] that set[**], then Foundation shall be free to grant such a sublicense to a Third Party [**]."

"(5) If Foundation makes a final decision, with respect to each and every Reversion Candidate, that it has no interest in performing or having performed (including through a sublicensee), at such time or at any point in the future, any further research, Development, or commercialization upon such Reversion Candidate pursuant to the Reversionary License, then it shall provide written notice thereof to Company, and Company shall be entitled to [**] and this Agreement on written notice to Foundation."

13. Clinical Trials and Access to Materials. The terms and conditions of this Second Amendment Section 13 shall apply equally to each Licensee as if such Licensee were Company, shall be included in the agreement pursuant to which Company grants rights to such Licensee with respect to any Drug Candidate or Product, and Foundation shall be a third party beneficiary with respect to such terms and conditions and shall have the right to take action directly against such Licensee if such Licensee fails to comply with such terms and conditions.

(a) SMAF Clinical Trials Advisory Committee. Foundation shall have the right, but not the obligation, to create a committee of experts to advise Foundation and Company on clinical trials and expanded access with respect to Development Candidates and Products (the "SMAF Clinical Trials Advisory Committee"). Such SMAF Clinical Trials Advisory Committee shall consist of such individuals as Foundation may designate, but shall include at least one clinical investigator with experience in the Field, [**]. The SMAF Clinical Trials Advisory Committee shall have, as one of its principal mandates, the responsibility of balancing (i) the rapid and efficient Development and commercialization of Development Candidates and Products for the benefit of all potential patients in the Field and (ii) the appropriateness, based on available safety and efficacy information with respect to such Development Candidates and Products, of providing access to such Development Candidates or Products to individual patients via the extension protocols to Company Clinical Trials or expanded access programs further

described in Second Amendment Sections 13(b) and 13(c). Such SMAF Clinical Trials Advisory Committee may establish its own procedures for meetings and decision-making.

(b) Company Clinical Trials.

(1) Foundation shall have the right, but not the obligation, to assist with patient recruitment for any Company Clinical Trial involving SMA patients by (i) referring to Company (or, at Company's request, referring directly to any clinical investigator at a clinical trial site for the applicable Company Clinical Trial) up to [**] SMA patients meeting the enrollment criteria for the applicable Company Clinical Trial and identified by Foundation or its designee, and/or (ii) proposing up to [**] clinical trial sites with access to appropriate patient populations for such Company Clinical Trial. Company shall use Commercially Reasonable Efforts to enable such patients to be enrolled in such Company Clinical Trial consistent with the applicable enrollment criteria, protocol, and target patient number for such Company Clinical Trial (it being understood that such patients should be given priority over other patients who are equally qualified to participate in such Company Clinical Trial, provided that the final decision regarding such enrollment is made by the clinical investigator and/or clinical trial site personnel of the investigating institution), and to contract with such clinical trial sites for such Company Clinical Trial. If Foundation, in its sole discretion, determines not to assist in patient recruitment for any Company Clinical Trial, then it shall so inform Company and Company shall assume all responsibility for patient recruitment and selection of clinical trial sites.

(2) Each time that Company commences the drafting of a clinical trial protocol for a Development Candidate or Product and at reasonable times thereafter, Company will discuss with Foundation Company's plans for making such Development Candidate or Product available to participants in such clinical trial after the completion of such trial. If mutually agreed by the parties based on such discussions, or if recommended by the SMAF Clinical Trials Advisory Committee in its sole discretion, Company will submit to the appropriate Regulatory Agency a suitable extension protocol and corresponding informed consent form providing for administration of such Drug Candidate or Product for at least [**] beyond the term provided for in a particular Company Clinical Trial. Company shall use Commercially Reasonable Efforts to obtain the applicable Regulatory Agency's approval of such extension protocol and informed consent and subsequent approval from the Institutional Review Boards at the locations where such Company Clinical Trial is being conducted; *provided, however*, that the proposed [**] period for such extension protocol may be shortened based on the request or advice of the applicable Regulatory Agency. Upon receipt of such approvals, Company shall provide, in accordance with the approved extension protocol, such Development Candidate or Product to those SMA patients who enrolled in such Company Clinical Trial pursuant to this Second Amendment Section 13(b) and wish to continue to receive such Development Candidate or Product after the completion of such Company Clinical Trial (such patients, the "Enrollees"). For so long as Company is continuing to develop or seek approval from a Regulatory Agency for such Development Candidate or Product, and subject either to mutual agreement of Company and Foundation or to the recommendation of the SMAF Clinical Trials Advisory Committee in its sole discretion, Company shall use commercially reasonably efforts to obtain approval for an amended or new extension protocol providing for continued administration of such Development Candidate or Product to the Enrollees, and Company shall

provide such Development Candidate or Product to the Enrollees in accordance with any such approved protocol. In any case in which Company, [**], does not concur in the decision to commence or continue any extension protocols pursuant to this Second Amendment Section 13(b)(2), then Company's obligations to assist with such extension protocols and continue to supply such Development Candidate or Product to Enrollees shall [**], directly or indirectly, [**], and [**] of Development Candidate or Product to Enrollees.

(3) If Company stops Developing or seeking approval from a Regulatory Agency of a Development Candidate or Product for which it filed an extension protocol pursuant to Second Amendment Section 13(b)(2), and either the parties mutually agree or the SMAF Clinical Trials Advisory Committee in its sole discretion (but having considered any safety issues) recommends that the Enrollees continue to have access to such Development Candidate or Product for a longer period than provided for in any existing extension protocol submitted by Company with respect to such Development Candidate or Product, then upon Foundation's request, Company shall facilitate Foundation's efforts to arrange for prolonged continued access to such Development Candidate or Product for some or all of the Enrollees by taking all reasonable actions requested by Foundation (consistent with the SMAF Clinical Trials Advisory Committee's recommendations, if applicable), including without limitation: (i) either (1) transferring Company's IND for such Development Candidate or Product to Foundation or its designee or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Company's IND or Drug Master File or equivalent regulatory filing (as applicable) so that Foundation or its designee can submit its own IND with respect to such continued access; (ii) providing (for the shorter of [**] months or the amount of time necessary for Foundation or its designee to establish an alternative supply of equivalent clinical grade product) such Development Candidate or Product to Foundation or its designee for administration to such Enrollees in accordance with any extension protocol for which Foundation or its designee has obtained approval from the FDA or the applicable Agency; (iii) assisting Foundation or its designee with obtaining an alternative, equivalent clinical grade supply of such Development Candidate or Product by (1) facilitating Foundation's or its designee's negotiation of a supply agreement with Company's manufacturer of such Development Candidate or Product or (2) providing

technology transfer and other technical assistance reasonably requested by Foundation to enable Foundation or its designee to manufacture such Development Candidate or Product; and (iv) providing Foundation with a non-exclusive, fully paid, sublicensable license under Company Technology and Data, and solely to the extent reasonably necessary for Foundation to exercise its rights under the foregoing license, to Company Base IP (*provided*, that the license granted hereunder to Foundation [**] or [**] or [**] to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide continued access to such Development Candidate or Product in accordance with this Second Amendment Section 13(b)(3). In any case in which Company, [**], does not concur in the decision to commence or continue any extension protocols pursuant to this Second Amendment Section 13(b)(3), then Company's obligations to assist with such extension protocols and continue to supply such Development Candidate or Product to Enrollees shall [**], directly or indirectly, [**], and [**] of Development Candidate or Product to Enrollees. In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with

respect to losses arising out of or related to the activities contemplated under this Second Amendment Section 13(b)(3). Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request.

(c) Expanded Access Program.

(1) At such a time as the parties mutually agree, or the SMAF Clinical Trials Advisory Committee in its sole discretion determines, that results from Company Clinical Trials and other Development activities with respect to the applicable Development Candidate or Product support expanded access to such Development Candidate or Product for patients with SMA, then Company and Foundation shall cooperate to establish such an expanded access program in which at least [**] SMA patients identified by Foundation who do not meet the enrollment criteria for a particular Company Clinical Trial (whether or not such Company Clinical Trial is directed to SMA patients) for such Development Candidate or Product (such patients, the "Patients") may gain access to such Development Candidate or Product. Company agrees that at its earliest reasonable opportunity following the commencement of such cooperation (e.g., at a meeting with FDA), Company will inquire about the feasibility of an expanded access protocol for such Drug Candidate or Product for SMA purposes and will invite a designee of Foundation with appropriate medical or regulatory experience to participate in discussions with the FDA regarding the establishment and maintenance of such expanded access program. In connection with such expanded access program, at Foundation's request and consistent with any recommendation made by the SMAF Clinical Trials Advisory Committee, Company will either (i) submit to the FDA a protocol that is reasonably acceptable to Foundation and calls for administering such Development Candidate or Product to the Patients or (ii) notify Foundation that it will not be making such a submission and facilitate the submission and approval of such a protocol by the Foundation or its designee.

(2) If Company chooses option (i) above, then it shall use Commercially Reasonable Efforts to obtain approval of such protocol and upon receipt of such approval, it shall provide such Development Candidate or Product to the Patients in accordance with the approved protocol; *provided*, that the parties shall engage in good faith negotiations with respect to [**].

(3) If Company chooses option (ii) above, then Company shall facilitate Foundation's efforts to arrange for such expanded access program for such Development Candidate or Product for the Patients by taking all reasonable actions requested by Foundation, in each case [**], including without limitation: (1) either (1) allowing the expanded access program to be performed pursuant to Company's IND (in which case Foundation or its designee shall provide Company with all data arising from and other information with respect to such expanded access program that is necessary or reasonably useful for Company to fulfill its obligations as the IND holder) or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Company's IND or Drug Master File or similar regulatory filing (as applicable) so that Foundation or its designee can file its own IND with respect to such expanded access program; (ii) providing such Development Candidate or Product to an appropriate designee of Foundation for administration to the Patients in accordance with any expanded access protocol for which Foundation or its designee has obtained approval from the FDA [**]); and (iii) providing Foundation with a non-

exclusive, fully paid, sublicensable license under Company Technology and Data, and solely to the extent reasonably necessary for Foundation to exercise its rights under the foregoing license, to Company Base IP (*provided*, that the license granted to Foundation hereunder [**] or [**] or [**] to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide such expanded access to such Drug Candidate or Product in accordance with this Second Amendment Section 13(c)(3). In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with respect to losses arising out of or related to the activities contemplated under this Second Amendment Section 13(c)(3). Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request.

(d) Indemnification by Foundation. In connection with the foregoing Second Amendment Sections 13(b)(3) and 13(c)(3), Foundation hereby agrees to save, defend, indemnify and hold harmless Company, its trustees, officers, employees and agents (each, a "Company Indemnitee") from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expenses and attorneys' fees ("Company Losses"), to which a Company Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Company Losses arise directly or indirectly out of (a) [**] or [**] of any Development Candidate or Product by Foundation, its Affiliate(s) or Licensee(s) pursuant to Second Amendment Sections 13(b)(3) or 13(c)(3), or (b) the breach of this Agreement by Foundation or the gross negligence or willful misconduct of Foundation pursuant to Second Amendment Sections 13(b)(3) or 13(c)(3), except in each case to the extent such Losses result from (x) the breach of this Agreement by Company or the gross negligence or willful misconduct of any Company Indemnitee, or (y) the activities of Company or its agents or employees in connection with any Development Candidate or Product. The obligations of Foundation under this Second Amendment Section 13(d) are conditioned upon Company's delivery of written notice to Foundation of any potential Company Losses promptly after Company becomes aware of such potential Company Losses. Foundation shall have the right to assume the defense of any suit or claim related to Company Losses if it has assumed responsibility for the suit or claim in writing. If Foundation defends the suit or claim, Company may participate in (but not control) the defense thereof at its sole cost and expense but Company may not settle such suit or claim without the prior written consent of Foundation, not to be unreasonably withheld.

(e) Clinical Trial/CRO Agreements. In connection with the foregoing Second Amendment Sections 13(b)(3) and 13(c)(3), Foundation hereby agrees that under any circumstance in which Foundation is contracting directly with clinical trial sites, clinical investigators, and contract research organizations ("CROs"), it will use as the basis for its negotiations [**], and will use Commercially Reasonable Efforts to secure terms with respect to publication, confidentiality, intellectual property (which shall be [**], as the case may be, [**]), and indemnification substantially similar to those routinely obtained by Company with respect to such an agreement, and naming the Company as a third-party beneficiary.

14. Patents. Section 6.2 of the Agreement (captioned "Patent Filings") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"6.2. Patent Filings.

"(a) Company shall control the filing, prosecution and maintenance of all Patents on Company Technology at its sole expense, which expense shall be included as part of Company's contribution to the Research Project and not payable or reimbursable by Foundation; *provided*, that Foundation shall have reasonable rights of comment and consultation on all such filing, prosecution and maintenance activities; and *provided further* that with respect to initial filings claiming the composition of matter, method of use, or process for manufacturing small molecules, Foundation's review shall be confined to specific individuals reasonably acceptable to Company.

"(b) Subject to the prior written consent of Foundation, such consent not to be unreasonably withheld, delayed or conditioned, Company shall have the right to disclose, in connection with the filing, prosecution or maintenance of any Patents on Company Technology filed by it pursuant to this Agreement, any Confidential Information to the extent reasonably necessary to support and enable the claims of any application with respect to such Patents, or to maintain or enforce any such issued Patents. If, with respect to a specific filing or other document to be submitted to a governmental or quasi-governmental authority in connection with an issued Patent or application for a Patent, Foundation has reviewed and commented on such filing or document pursuant to Section 6.2(a) of the Agreement and raised no objections to the use of Confidential Information, then such consent will be deemed to have been granted for such filing or document.

"(c) Notwithstanding the foregoing Sections 6.2(a) and 6.2(b) of the Agreement, if Company grants the Reversionary License to Foundation pursuant to Section 6.1(c)(2) of the Agreement with respect to a Reversion Candidate or Reversion Product, then Foundation (or its designee) shall have the rights and obligations of Company under Sections 6.2(a) and 6.2(b) of the Agreement (substituting "Foundation" for "Company" as the context requires) with respect to Patents exclusively licensed to Foundation pursuant to such Reversionary License; *provided*, *however*, that [**], and [**] of the Agreement.

"(d) Each of Company and Foundation shall execute all papers and instruments, and require its employees and contractors to execute all papers and instruments, so as to enable the other party to exercise the rights set forth in this Section 6.2 of the Agreement."

15. Confidentiality and Exceptions. Section 5.1 of the Agreement (captioned "Confidentiality") and Section 5.2 of the Agreement (captioned "Exceptions") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"5.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the parties, the parties agree that, during the Term and for a period of [**] years thereafter, each party (the ***"Receiving Party"***) will maintain in confidence all Confidential Information disclosed to it by the other party (the ***"Disclosing Party"***), provided that, with regard to Confidential Information which is trade secret information, such obligation shall extend thereafter until such information is

no longer a trade secret of the Disclosing Party. The Receiving Party may use the Confidential Information of the Disclosing Party only to the extent required to accomplish the purposes of this Agreement. The Receiving Party shall use at least the same standard of care as it uses to protect proprietary or confidential information of its own to ensure that its employees, agents, consultants and other representatives do not disclose or make any unauthorized use of the Disclosing Party's Confidential Information; provided, however, each party shall ensure that any such employees, agents, consultants and other representatives who are granted access to trade secrets or, prior to publication, to other potentially patentable matter for which patent protection has been or is planned to be sought, arising from the DC Research shall sign written agreements containing confidentiality obligations substantially similar to those set forth in this Agreement except that the duration of such confidentiality obligations for consultants may be less than the duration set forth in this Agreement provided that the duration shall be for a minimum of [**] years from the date of disclosure. Each party will promptly notify the other upon discovery of any unauthorized use or disclosure of the other party's Confidential Information.

"5.2 Exceptions. The obligations of non-disclosure and non-use contained in Section 5.1 will not apply to the extent that it can be established by the Receiving Party by competent proof that such Confidential Information: (a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement; (d) is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or (e) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others."

16. Publications, Presentations and Public Disclosures. Section 5.4 of the Agreement (captioned "Publication") and Section 5.5 of the Agreement (captioned "Publicity; Regulatory Disclosures") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"5.4. Scientific and Medical Publications and Presentations.

"(a) Company and Foundation each acknowledge the other party's interest in publishing or presenting certain results of the Research (including but not limited to the DC Research) to obtain recognition within the scientific community and to advance the state of scientific knowledge and enhance the progress of research in the Field, in all cases in a manner consistent with existing obligations to Third Parties and scientific and industry standards for the research, development and commercialization of small molecules for the treatment, mitigation or prevention of disease. Each party also recognizes their mutual interest in obtaining Patents in support of Products, and the need for such publications or presentations to be strictly monitored to prevent any adverse

effect from premature publication or dissemination of results of the activities hereunder. Consequently, the JSC shall establish reasonable policies and procedures with respect to scientific publications and presentations that balance the foregoing interests, and once established both parties shall be bound by such policies and procedures, and may establish a separate publication committee to administer such policies and procedures. In the event no such procedures and policies are established by the JSC, either party, its employees or consultants wishing to make a publication in a scientific or medical journal or a presentation or similar oral disclosure made at a scientific or medical conference without obligation of confidentiality relating to work performed as part of the Research (the "Publishing Party") shall transmit to the other party (the "Reviewing Party") a copy of the proposed written publication or a written detailed description of the proposed oral disclosure at least [**] days prior to submission or disclosure (or, in the case of Third Party agreements, such shorter period as required by such Third Party agreement) prior to submission for publication or presentation. The Reviewing Party shall have the right (a) to make modifications to the publication for accuracy or intellectual property reasons, and (b) to obtain a delay in publication or presentation of up to [**] days (or, in the case of Third Party agreements, such shorter period as required by such Third Party agreement) in order to enable patent applications or similar applications protecting rights in such information to be filed, and each party shall have the right to prohibit disclosure of any of its Confidential Information (except as otherwise provided in Section 6.2 of the Agreement) in any such proposed publication or presentation. Notwithstanding the foregoing, in no event shall any publication, presentation, or other public disclosure disclose the chemical structure of any Lead Candidate, Drug Candidate, Development Candidate, or Product absent specific permission from the JSC. In any permitted publication or presentation by a party, the other party's contribution shall be duly recognized, and authorship shall be determined in accordance with customary practice in the scientific or medical field.

(b) Company shall provide in each Research Report a summary section which is suitable for immediate public disclosure and the Foundation may release copies of such portions of each Research Report and supporting Data other than chemical structures to any Third Party investigator who requests such material from the Foundation in writing; *provided, however,* that said Third Party investigator first executes Company's non-disclosure agreement that the Company provides to the Foundation for such purpose (it being understood that such non-disclosure agreement will not prohibit said Third Party investigator from applying his or her knowledge of the Data to further SMA research and/or to treatment of SMA patients, but will prohibit him or her from transferring such Data except as incidental and necessary to treating SMA patients).

(c) The parties acknowledge that during the course of research, development and commercialization of Products, it may be necessary to enter into agreements with Third Parties that require different standards for publication and presentation of research results relating to the Research. Notwithstanding Section 5.4(a) of the Agreement, the party conducting research, development or commercialization of Products may enter into agreements with academic, government, nonprofit or similar entities which allow principal investigators and other external researchers to publish or present the results of

their research on terms inconsistent with Section 5.4(a) of the Agreement; provided, that each party entering into such agreements shall use Commercially Reasonable Efforts to include provisions reasonably consistent with and similar to those appearing in Section 5.4(a) of the Agreement in such Third Party agreements.

"5.5 Publicity; Regulatory Disclosures. In connection with the execution of the Second Amendment, the parties shall jointly issue one or more press releases, the contents of which shall be mutually agreed. Except as otherwise required by law or regulation, or as permitted pursuant to Section 5.4 of the Agreement, neither party shall issue any additional press release or make any other public disclosure concerning this Agreement or the subject matter hereof without first providing the other party with a copy of the proposed release or public disclosure for review and comment, provided that such right of review and comment shall only apply for the first time that specific information is to be disclosed, and shall not apply to the subsequent disclosure of substantially similar information that has previously been disclosed. The party proposing to make the press release or other public disclosure shall give due consideration to any reasonable comments by the other party relating to such proposed press release or other public disclosure. The principles to be observed by the parties in press releases or other public disclosures with respect to this Agreement shall be: accuracy, compliance with applicable legal and regulatory requirements, the requirements of confidentiality under this Agreement and customary business practice in the biopharmaceutical industry for disclosures by companies comparable to Company. For the avoidance of doubt, either party may issue such press releases as it determines, based on advice of counsel, are reasonably necessary to comply with laws or regulations (including regulations of non-governmental regulatory bodies) or for appropriate market disclosure. It is understood, however, that unless required by law or regulation, the parties shall not disclose the specific financial terms and conditions of this Agreement in any press release or other public disclosure. In addition, if a public disclosure is required by law or regulation, including without limitation in a filing with the United States Securities and Exchange Commission, the disclosing party shall provide copies of the proposed disclosure reasonably in advance of such filing or other disclosure for the non-disclosing party's prior review and comment and shall give due consideration to any reasonable comments by the non-filing party relating to such filing, including without limitation the provisions of this Agreement for which confidential treatment should be sought."

17. Baseball Arbitration.

(a) An arbitration under this Second Amendment Section 17 (a "Baseball Arbitration") shall be initiated by written notice of one party to the other and may only be initiated with respect to disputes which meet both the following criteria: (i) the dispute arises from matters within the jurisdiction of the JSC following escalation to the respective Chief Executive Officers of the parties as provided elsewhere in the Agreement, and (ii) Baseball Arbitration is explicitly specified as the method for resolving such dispute pursuant to the Agreement.

(b) The Baseball Arbitration shall be held in a location mutually agreeable to the parties, or if no such location can be agreed, in New York City, according to the then-current commercial arbitration rules of the American Arbitration Association ("AAA"), except to the extent such rules are inconsistent with this Second Amendment Section 17.

(c) The Baseball Arbitration will be conducted by one (1) arbitrator who shall be reasonably acceptable to the parties and who shall be appointed in accordance with AAA rules. If the parties are unable to select an arbitrator within [**] days of the notice that initiated the Baseball Arbitration, then the arbitrator shall be appointed in accordance with AAA rules. Any arbitrator chosen hereunder shall have educational training and industry experience sufficient to demonstrate a reasonable level of scientific, financial, medical and industry knowledge relevant to the particular dispute.

(d) Within [**] days after the selection of the arbitrator, each party shall submit to the arbitrator and the other party a proposed resolution of the dispute that is the subject of the arbitration, together with any relevant evidence in support thereof (the "Proposals"). Within [**] business days after the delivery of the last Proposal to the arbitrator, each party may submit a written rebuttal of the other party's Proposal and may also amend and re-submit its original

Proposal. The parties and the arbitrator shall meet within [**] business days after the parties have submitted their final Proposals (and rebuttals, if any), at which time each party shall have [**] to argue in support of its Proposal. The parties shall [**]. Within [**] days after such meeting, the arbitrator shall select one of the final Proposals so submitted by one of the parties as the resolution of the dispute, but may not alter the terms of either final Proposal and may not resolve the dispute in a manner other than by selection of one of the submitted final Proposals. If a party fails to submit a Proposal within the initial [**] day time frame set forth in the first sentence of this Second Amendment Section 17, the arbitrator shall select the Proposal of the other party as the resolution of the dispute. Any time period set forth in this Second Amendment Section 17 may be extended by mutual agreement of the parties.

(e) No arbitrator shall have the power to award punitive damages under this Agreement regardless of whether any such damages are contained in a Proposal, and such award is expressly prohibited. The proceedings and decisions of the arbitrator shall be confidential, final and binding on the parties. Judgment on the award so rendered may be entered in a court having jurisdiction thereof.

(f) [**] the costs of such Baseball Arbitration.

18. Miscellaneous.

(a) Compounds not Selected: During the Second Amendment Term and for the [**] period thereafter, should Company require additional funds for the conduct of research or Development in the Field of any Lead Candidate that was tested in the course of the DC Research but was not selected as a Reversion Candidate (such research and Development, "Non-DC Research"), Foundation will be consulted and provided the opportunity to fund such Non-DC Research in whole or in part prior to any fundraising efforts for such Non-DC Research. Should Company identify an opportunity for agreement with any Third Party or Third Parties with respect to such Non-DC Research during such [**] period, it will provide reasonable advance

notice to Foundation, and the parties will negotiate in good faith (involving such Third Party or Third Parties as appropriate) to develop a structure that supports such additional funding, based on the following principles: (a) entities co-funding such Non-DC Research should share information on the Non-DC Research with each other, subject to appropriate confidentiality provisions, (b) governance with respect to co-funded Non-DC Research should be via a joint steering committee including representatives of Foundation, Company, and any Third Parties, and (c) entities that have provided funding to such co-funded Non-DC Research should have an opportunity (subject to compliance with the terms of their respective funding agreements) to continue their support of such Non-DC Research. For clarity, Company's obligations under this Second Amendment Section 18(a) shall in no way limit Company's ability to engage in general fund-raising activities and to enter into agreements relating thereto.

(b) Additional Testing of Reversion Candidates. After the end of the Research Term and selection of one or more Development Candidates, the JSC (at the request of either party) shall consider whether further research or pre-clinical Development on any Reversion Candidate that is not a Development Candidate is advisable for the purposes of enhancing the utility of such Reversion Candidate as a potential back-up compound or next-generation Product. If deemed advisable by the JSC, and subject to agreement between the parties with respect to funding, Company shall make such Reversion Candidates available for such further research or pre-clinical Development under the terms of a commercially reasonable materials transfer agreement (or a more comprehensive agreement agreed by the parties with respect to such Third Party, which shall include commercially reasonable terms with respect to materials transfer).Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any compound become the property of such Third Party, nor shall any such compound become subject to royalty or other reach-through payment obligations to such Third Party as a result of such testing by such Third Party, and shall also require that a summary of the results of the research be provided to the Company. Company shall share all such research results with the JSC.

(c) Testing by Foundation Partners. Upon the Foundation's request, and under the supervision of the JSC with respect to design of the testing to be done and selection of appropriate compounds and (only during the Research Term) consistent with the then-current Research Plan, Company shall provide reasonable quantities of compounds synthesized or tested during the DC Research ("Research Compounds") to other Foundation partners for testing on a blinded basis in assays already being run by such Foundation partner, provided that (i) after the end of the Research Term, such testing shall be limited to Reversion Candidates and shall not include, without the prior written consent of Company, any Development Candidate that is, at such time, the subject of a Company Clinical Trial, (ii) the Foundation or such Foundation partner shall disclose the results of such screening to Company and (iii) such testing shall be performed pursuant to a separate materials transfer agreement reasonably acceptable to Company and negotiated in good faith by the parties prior to provision of any Research Compounds or related information, which agreement shall contain reasonable and customary terms to protect the parties' respective intellectual property rights. Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any Research Compound become the property of the Foundation partner, nor shall any Research Compound become subject to royalty or other reach-through payment obligations to the

Foundation partner as a result of such testing by such Foundation partner, and shall also require that a summary of the results of the research be provided to the Foundation. Foundation shall share all such research results with the JSC on a regular basis.

(d) Financial Reporting. For so long as it is not a publicly-traded company, (i) Company shall provide Foundation, within [**] days after the end of each of the first three quarters of the fiscal year of the Company, with a copy of the financial report for such quarter that Company generates for its investors, and (ii) Company shall use best its efforts to provide within [**] days, but in no event more than [**] days, after the end of the fiscal year of the Company, a copy of the annual audit report for such year that the Company generates for its investors. The financial reports provided pursuant to Second Amendment Section 18(d)(i) shall be prepared in accordance with generally accepted accounting principles consistently applied, and duly certified (subject to year-end audit adjustments) by the chief financial officer of the Company, and the annual audit report provided pursuant to Second Amendment Section 18(d)(ii) shall be duly certified by independent public accountants of recognized standing. [**], Company may request Foundation consider amending the provisions of this Second Amendment Section 18(d), and Foundation shall consider such request in good faith.

(e) Representations, Warranties and Covenants. Each party hereby represents, warrants and covenants to the other that (i) it has the authority and right to enter into this Second Amendment and to perform its obligations hereunder and (ii) it has not granted as of the Second Amendment Effective Date, and will not grant during the Term (except as specifically allowed and consistent with the applicable terms of this Agreement), any assignment, license, covenant not to sue, option to obtain a license or other right, interest or benefit, exclusive or otherwise, to any Third Party relating to the DC Research or any Reversion Candidate, Drug Candidate, Development Candidate or Product that conflicts with or limits the rights granted to or exercisable by the other party hereunder.

(f) Designation of Third Party Representatives. In any case under this Agreement under which Foundation is entitled to designate a representative, such representative may be a person other than a Foundation employee provided the following criteria are met: (i) Foundation identifies such person in writing to

Company, [**], and requests Company's written consent for such addition, such consent not to be unreasonably withheld or delayed and to be based solely on objective written criteria determined by the JSC within [**] months after the Second Amendment Effective Date, which criteria shall vary depending upon the information to which such person is anticipated to have access to in the course of his or her service as such representative; and (ii) such person signs a confidentiality agreement with Company substantially in the form attached as Exhibit SA-7. The JSC shall also establish policies and procedures regarding continuing disclosure obligations for such representatives with respect to conflicts of interest within [**] months of the Second Amendment Effective Date. A breach by a designated representative of Foundation of such conflict of interest policies, or of the confidentiality agreement between Company and such representative, shall entitle Company to terminate such representative effective upon written notice by Company to Foundation stating the grounds for such termination.

(g) Corrective Amendments.

(i) The following Section of the Agreement is cancelled and of no further force and effect: Section 1.20 of the Agreement, the last sentence of Section 2.4(b) of the Agreement, the Option defined in First Amendment Section 2, and First Amendment Section 8.

(ii) Section 2.8 of the Agreement (captioned "Subcontracts") is amended and restated as follows:

"2.8 Subcontracts. Company may perform some of its obligations under the Research Plan through one or more subcontractors, provided that (a) the Research Plan calls for such activities to be subcontracted, (b) none of the rights of either party hereunder are diminished or otherwise adversely affected as a result of such subcontracting, and (c) Company will at all times be responsible for the performance and, except as otherwise agreed by the parties in writing, payment of such subcontractor. In determining whether any Company obligations under the Research Plan will be performed in-house or by a Third Party subcontractor, Company shall take into consideration Company's then-current capabilities, the relative efficiency of utilizing such internal capabilities versus Third Party services and guidance from the JSC."

(iii) Section 6.1(a) of the Agreement (captioned "Data") is amended and restated as follows:

"(a) Data. Company shall solely own all Data."

(iv) Section 6.4 of the Agreement (captioned "No Other License") is amended and restated as follows:

"6.4 No Other License. Other than any licenses granted pursuant to Section 6.1(c) and Second Amendment Section 3 or 13, no license is granted or implied with respect to any Company Technology, Company Base IP or Data for any use."

(v) Section 7.1 of the Agreement (captioned "Term") is amended and restated as follows:

"7.1 Term. The term of this Agreement (the "Term") shall commence on the Effective Date and shall continue until the earliest of: (a) Foundation's receipt of the Repayment Amount in full (including any subsequent payments due on account of [**]); (b) if Foundation exercises a Buy-Out Right, Company's receipt of all payments due pursuant to Section 6.1(c)(3) of the Agreement; (c) if Foundation obtains a Reversionary License pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), Company's receipt of all payments due pursuant to Section 6.1(c)(4) of the Agreement; (d) if Foundation obtains a Reversionary License other than as a result of the exercise of a Buy-Out Right or pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), the expiration of the last-to-expire Patent licensed to Foundation pursuant to such Reversionary License; or (e) the effective date of any termination in

accordance with this Article 7. For clarity, a Special Termination shall not terminate the term of this Agreement."

(vi) The last sentence of Section 7.3 of the Agreement (captioned "Termination Upon Principal Scientist's Unavailability") is amended and restated as follows: "In the event of a termination of this Agreement pursuant to this Section 7.3 of the Agreement, and notwithstanding any other provision of this Agreement to the contrary (including but not limited to Section 7.4 of the Agreement), only the provisions of Sections 4.7, 6.1(a), 6.1(b), 6.1(c)(2), 6.2(a), 6.2(b), 6.2(d), 7.3, 7.5 of the Agreement, Articles 1, 5, 8, and 9 of the Agreement and Sections 1, 4(b)(ii), and 18(a) of the Second Amendment will survive such termination of this Agreement."

(vii) Section 7.4 of the Agreement (captioned "Consequences of Expiration or Termination") is amended and restated as follows:

"7.4 Consequences of Expiration or Termination. Expiration or termination of this Agreement will not relieve the parties of any obligation accruing prior to such expiration or termination. Except as otherwise provided in Section 7.3 of the Agreement in the case of a termination pursuant to its terms, and notwithstanding any other provision of this Agreement to the contrary, only the provisions of Sections 4.3, 4.4, 4.5, 4.6, 4.7, 7.4, and 7.5 of the Agreement, and Articles 1, 5, 6 (to the extent applicable), 8 and 9 of the Agreement and Sections 1, 4(b)(ii), and 18(a) of the Second Amendment will survive expiration or termination of this Agreement."

(viii) The second sentence of Section 7.5 of the Agreement is amended and restated as follows: "The parties agree that the Foundation, to the extent it receives a license pursuant to Section 6.1(c) of the Agreement or Second Amendment Section 3 or 13, will retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code."

(ix) Section 9.7 of the Agreement (captioned "Notices") is amended and restated as follows:

"9.7 Notices. All notices and other communications provided for hereunder shall be in writing and shall be mailed by first-class, registered or certified mail, postage paid, or delivered personally, by overnight delivery service or by facsimile, with confirmation of receipt, addressed as follows:

"If to Foundation:

"Spinal Muscular Atrophy Foundation
"888 Seventh Avenue, Suite 400
"New York, NY 10019
"Fax: (212) 247-3079
"Attention: Ms. Cynthia Joyce, Executive Director

"With a copy to:

"Cooley Godward Kronish LLP
"4401 Eastgate Mall
"San Diego, CA 92121
"Fax: (858) 550-6420
"Attention: Matthew Browne, Esq.

"If to Company:

"PTC Therapeutics, Inc.
"100 Corporate Court
"South Plainfield, NJ 07080-2449
"Fax: 908-222-1128
"Attention: Legal Department

"With an email copy to: legal@ptcbio.com

"Either party may by like notice specify or change an address to which notices and communications shall thereafter be sent. Notices sent by facsimile shall be effective upon confirmation of receipt, notices sent by mail or overnight delivery service shall be effective upon receipt, and notices given personally shall be effective when delivered."

19. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Second Amendment and the terms of the Agreement or the First Amendment, the terms of this Second Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Second Amendment.

IN WITNESS WHEREOF, the parties have executed this Second Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.

/s/ Stuart Peltz
By: Stuart Peltz
Title: President & CEO

SPINAL MUSCULAR ATROPHY FOUNDATION

/s/ Florence A. Eng
By: Florence A. Eng
Title: President

EXHIBIT SA-1

Research Plan for DC Research

[See following for Research Plan and related budget]

FTE Rates

FTE Category	FTE Rate (Annual)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

DC Research Plan
Exhibit SA-1

[**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of seven pages were omitted. [**]

Note: This draft budget is based on slides presented to SMAF in November 2008 that contemplated funding by SMAF commencing in December 2008. The dates and numbers in this draft will need to be adjusted depending on the date we actually sign the contract.

DC Research Budget

Draft of December 31, 2008

Ours is a New Way of Looking

MONTHS 1-12: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY SMAF

Employee Type	12/08	1/09	2/09	3/09	4/09	5/09	6/09	7/09	8/09	9/09	10/09	11/09	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - SMA ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 13-24: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY SMAF

Employee Type	12/09	1/10	2/10	3/10	4/10	5/10	6/10	7/10	8/10	9/10	10/10	11/10	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total – SMA ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 1-12: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY PTC

Employee Type	12/08	1/09	2/09	3/09	4/09	5/09	6/09	7/09	8/09	9/09	10/09	11/09	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - PTC ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 13-24: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY PTC

Employee Type	12/09	1/10	2/10	3/10	4/10	5/10	6/10	7/10	8/10	9/10	10/10	11/10	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - PTC ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

SMA PROJECT BUDGET SUMMARY

	Gap Period July 2008 -Nov 2008	Year One Dec 2008 - Nov 2009	Year Two Dec 2009 - Nov 2010	Total
SMA Request	[**]	[**]	[**]	[**]
PTC Share	[**]	[**]	[**]	[**]
Total Investment	[**]	[**]	[**]	[**]

PTC Therapeutics, Inc. ©2009 -Slide 5

EXHIBIT SA-2

Format for DC Research Reports

[See following]

Project: XYZ

Date:
Period Covered:
Project Objective:

SMAF Logo

Major Accomplishments

Key Issues - Plans to Address Them

Next Major Milestones and Dates

EXHIBIT SA-3

Product Profile Format for other SMA Efforts

[See following]

Exhibit SA-3: Lead Profile Status Report (Unfunded)

Attributes	Other Effort(1)
Potency ($EC_{1.5X}$)	
Increase SMN protein level in cells (fold)	
Target selectivity	
Selectivity Index ($CC_{50}/EC_{1.5X}$)	
Metabolism (%loss in 1hr)	
Route of administration	
PK (e.g. exposure)	
hERG inhibition (% at 5 µM)	
P450 inhibition (IC_{50})	
Increase SMN expression in a mouse model	

(1) Effort by PTC in SMA field not covered under the collaboration agreement

Confidential - PTC Therapeutics, Inc. © 2009 - Slide 0

EXHIBIT SA-4A

Development Deadline Document

This Development Deadline Document is an Exhibit to the Sponsored Research Agreement, as amended (the "Agreement"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), and describes deadlines for Development as further specified in that Agreement. Capitalized terms used but not otherwise defined in this Development Deadline Document shall have the meanings set forth in the Agreement.

Activity/Milestone	Deadline (months)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	

EXHIBIT SA-4B

Company's Anticipated Development Activities and Goals

This Exhibit SA-4B to the Sponsored Research Agreement, as amended (the "Agreement"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), describes the Company's expectations, as of the Second Amendment Effective Date, of the activities

required to obtain Regulatory Approval and its goal timelines for completing such activities. Capitalized terms used but not otherwise defined in this Exhibit SA-4B shall have the meanings set forth in the Agreement.

Activity/Milestone	Goal (months)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	

EXHIBIT SA-5

Foundation Representatives with Access to Shared Electronic Collaboration Space

[**]

EXHIBIT SA-6

Foundation Representatives with Access to Sensitive Data in Shared Electronic Collaboration Space

[**]

EXHIBIT SA-7

Form of CDA for Foundation Representatives

[See following]

CONFIDENTIALITY AGREEMENT

This Agreement is made as of , 20 , by and between PTC Therapeutics, Inc., having an address of 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ, 07080 (the "Company"), and ("Recipient"), a designated representative of the Spinal Muscular Atrophy Foundation ("Foundation") pursuant to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended (the "SRA").

1. Background. The Recipient will serve as the designated representative of Foundation pursuant to Section of the SRA.

2. Proprietary Information. As used in this Agreement, the term "Proprietary Information" shall mean all confidential or proprietary information or materials of the Company, whether disclosed in writing, orally, or visually, including, without limitation, technical information, including inventions, methods, plans, processes, specifications, characteristics, chemical structures, raw data, know-how, experience, and trade secrets; developmental, marketing, sales, operating, performance, and cost information; and all record bearing media containing or disclosing the foregoing information, including business plans, patents and patent applications, grant applications, notes, and memoranda, or drafts of any of the foregoing, whether in writing or presented, stored or maintained in or by electronic, magnetic, or other means.

3. Disclosure of Proprietary Information. The Recipient shall hold in confidence, and shall not disclose to any person, any Proprietary Information disclosed to Recipient by the Company or the Foundation except as specifically permitted herein. The Recipient shall use such Proprietary Information only to accomplish the purposes of the SRA and shall not use or exploit such Proprietary Information for his own benefit or the benefit of another (except for the benefit of the Foundation as contemplated by the SRA) without the prior written consent of the Company. The Recipient may disclose Proprietary Information received by him under this Agreement only (i) to the Foundation and its Affiliates subject to the obligations of confidentiality specified in the SRA; or (ii) to those other representatives of Foundation who have a need to know such Proprietary Information in the course of the performance of their duties and who are bound by written agreement to protect the confidentiality of such Proprietary Information in accordance with terms set forth in the SRA.

4. Limitation on Obligations. The obligations of the Recipient specified in Section 3 above shall not apply, and the Recipient shall have no further obligations hereunder, with respect to any Proprietary Information to the extent that such Proprietary Information;

(a) is generally known to the public at the time of disclosure or becomes generally known through no wrongful act on the part of the Recipient;

(b) is in the Recipient's possession at the time of disclosure to Recipient other than as a result of prior disclosure by the Company or Foundation or a breach of any legal obligation by Recipient or third party;

(c) is in the Foundation's possession at the time of disclosure to Foundation other than as a result of prior disclosure by the Company or a breach of any legal obligation by Foundation or third party;

(d) becomes known to the Recipient through disclosure by a source other than the Company or Foundation, provided that such source has no duty of confidentiality to the

Company, whether direct or indirect, with respect to such Proprietary Information and has the legal right to disclose such Proprietary Information;

(e) is independently developed by the Recipient or Foundation without reference to or reliance upon the Proprietary Information, as can be documented by written records; or

(f) is required to be disclosed by the Recipient or Foundation to comply with applicable laws or governmental regulations, provided that the Recipient provides prior written notice of such disclosure to the Company and takes reasonable and lawful actions to avoid and/or minimize the extent of such disclosure.

5. Ownership of Proprietary Information. The Recipient agrees that the Company is and shall remain the exclusive owner of the Proprietary Information and all patent, copyright, trade secret, trademark and other intellectual property rights therein. No license or conveyance of any such rights to the Recipient is granted or implied under this Agreement.

6. Return of Documents. The Recipient shall, upon the request of the Company, turn over to the Foundation, or with the Foundation's prior written permission, return to the Company, all drawings, documents, materials and other tangible manifestations of the Proprietary Information received by the Recipient pursuant to this Agreement (and all copies and reproductions thereof) except that one copy of each may be retained by Recipient for the purposes of assuring compliance with the terms of this Agreement.

7. Term. The Recipient's obligations with respect to each item of Proprietary/Information shall extend until [**] years from the end of the Term of the SRA; provided, however, that with respect to trade secret information, Recipient's obligations shall further extend until such information no longer constitutes a trade secret of Company under applicable law.

8. Miscellaneous.

(a) The Company acknowledges that the Recipient is a representative of the Foundation and that Company has the confidentiality obligations set forth in Section 5.1 of the SRA with respect to any confidential or proprietary information of Foundation disclosed to Company by the Recipient.

(b) This Agreement supersedes all prior agreements, written or oral, between the Company and the Recipient relating to the subject matter of this Agreement. This Agreement may not be modified, changed or discharged, in whole or in part, except by an agreement in writing signed by the Company and the Recipient.

(c) This Agreement will be binding upon and inure to the benefit of the parties hereto and their respective heirs, successors and assigns.

(d) This Agreement shall be construed and interpreted in accordance with the laws of the State of New York, without giving effect to conflict of laws provisions.

(e) The provisions of this Agreement are necessary for the protection of the business and goodwill of the parties and are considered by the parties to be reasonable for such purpose. The Recipient agrees that any breach of this Agreement will cause the Company substantial and irreparable harm and, therefore, in the event of any such breach, in addition to other remedies which may be available, the Company shall have the right to seek specific performance and other injunctive and equitable relief.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their duly authorized representatives as of the date first above written.

PTC THERAPEUTICS, INC.

_____

Name:
Title:

RECIPIENT:

_____

Name:
Title:
Institutional Affiliation/Employer:

EXHIBIT SA-8

Foundation initial JSC and Joint Team Members

Foundation initial JSC members:

[**]

Foundation initial Joint Team members:

[**]

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AMENDMENT No. 3 TO SPONSORED RESEARCH AGREEMENT

This third amendment ("Third Amendment") to the Sponsored Research Agreement is effective as of the 1st day of January, 2011 (the "Third Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement (the "Agreement") dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007 and by the Second Amendment on May 1, 2009 (the "Second Amendment");

WHEREAS, the parties desire to further amend the Agreement in connection with continued research, beyond the [**] specified in the Second Amendment, on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Third Amendment, the parties agree as follows:

1. Continuing Research.

(a) By letter dated [**], Foundation called a special meeting of the JSC pursuant to Second Amendment Section 2(g) to address the Cost/Timeline Issue that a [**] by the [**] of [**] and that the [**] of $[**] before a [**] would be [**]. As contemplated by Second Amendment Section 2(g)(1) the JSC has agreed upon the Corrective Plan and related budget that are attached as Exhibits TA-1 and TA-2, respectively. Such Corrective Plan and related budget constitutes an amendment of the Research Plan and related budget. Company shall conduct the DC Research in accordance with the Agreement, as amended.

(b) In connection with adoption of the Corrective Plan, the JSC has also agreed upon the DC Criteria attached as Exhibit TA-3 and the parties have agreed to extend the DC Timeline Goal to [**] and the Research Term until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) [**] or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3. If a Development Candidate is not selected by the extended DC Timeline Goal, the parties shall have the right to call a special meeting of the JSC to address [**] in accordance with Second Amendment Section [**], including by agreeing upon [**], and if applicable thereafter, the rights specified in Section Amendment Section 2(h) and/or Second Amendment Section [**].

(c) Notwithstanding Second Amendment Section 2(d), Company shall be responsible for funding one hundred percent (100%) of the total overall cost of all DC Research performed on or after the Third Amendment Effective Date; provided, however, that Company shall have the ability to set its own budgets with respect to the conduct of the research after [**] so long as the Company's obligations under Section 2.5 of the Agreement (captioned "Performance Standards") are met. Company acknowledges and agrees that Foundation has paid all amounts due to Company pursuant to Second Amendment Section 2(d) and does not have any further

obligation to reimburse Company for any amounts incurred by Company, whether before or after the Third Amendment Effective Date, with respect to the DC Research. If Foundation decides in its discretion to engage [**] or other external contract research organizations ("CROs") or academic collaborators [**] to test, after the Third Amendment Effective Date, any compounds arising from the DC Research, Foundation shall be solely responsible for paying any amounts owed to [**] or such other CROs or academic collaborators in connection with such testing.

2. Governance. Second Amendment Section 5(d) is amended and restated in its entirety as follows:

"(d) Meetings and Decision-Making by the JSC — Before Proof-of-Concept. During the Research Term and through achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**], in person (with the location to be at Foundation's offices in New York City unless otherwise agreed by the Parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or the secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall meet in person to attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment; provided, however, that the following matters shall not be subject to such referral to Baseball Arbitration, : (i) [**]; (ii) any [**] described in Second Amendment Section [**] as [**] to or [**]; (iii) any changes to [**] for the [**] that would require [**] than contemplated in [**]; (iv) deciding whether to pursue [**] of this Second Amendment in the event of a [**]; and (v) any disputes referred to the CEOs pursuant to Second Amendment Section [**]. Disputes not subject to referral to Baseball Arbitration pursuant clauses (i) through (v) of the preceding sentence shall be resolved as follows: any dispute arising in the JSC with respect to clause (v) shall be decided by [**], and any disputes arising in the JSC with respect to clauses (i) through (iv) may only be resolved by mutual agreement of the parties."

3. Partnering Activities.

Second Amendment Section 10(c) is amended and restated in its entirety as follows:

"(c) If Company determines to actively pursue Collaboration Activities, whether at its own initiative or in response to inquiries from Third Parties, Company will first seek input from Foundation through a mutually-agreed team of Foundation representatives

(the "Foundation Partnering Team") on the nature, scope, and potential terms of a transaction arising in connection with such Collaboration Activities, as well as a rank-ordered summary list of preferred potential counterparties to such transaction. To the extent prepared by Company rather than received by Company from a potential counterparty, Company shall also provide the Foundation Partnering Team with an opportunity to review a draft term sheet and related materials in support of its proposed Collaboration Activities. The Foundation shall collect input from the Foundation Partnering Team on Company's overall approach to Collaboration Activities, as well as specific input on any term sheet or related materials provided to the Foundation Partnering Team, and shall promptly provide such input to Company. The initial mutually-agreed members of the Foundation Partnering Team are set forth on Exhibit TA-4. The Foundation may replace the outside counsel member of the Foundation Partnering Team with an alternative outside counsel chosen by the Foundation; such replacement will be effective upon notice to Company. The Foundation may replace any other member of the Foundation Partnering Team with an alternative individual chosen by the Foundation; such replacement will be effective upon PTC's written consent, which will not be unreasonably withheld or delayed."

Second Amendment Section 10(e) is amended and restated in its entirety as follows:

"(e) Prior to Company entering into a definitive written agreement with any Third Party in connection with Collaboration Activities, Company shall seek the review and approval of the Foundation by providing the members of the Foundation Partnering Team with a proposed final draft of the definitive written agreement, a summary (which may be oral or written) of the proposed transaction, including an overview of any items or terms subject to finalization in the draft provided, and the timely opportunity (which may include one or more in-person meetings) to discuss such draft and summary and answer the Foundation's questions with respect thereto. If required by the Company's confidentiality agreement with the potential counterparty, Company shall have the right to redact financial terms from such proposed final draft of the definitive written agreement. As promptly as reasonably possible, but in no event later then [**] business days following receipt by the Foundation Partnering Team members of such proposed final draft of the definitive written agreement and summary, Foundation shall either approve or deny such proposed transaction; *provided, however*, that if Company has otherwise complied with requirements of this Second Amendment Section 10, Foundation shall only be entitled to deny such proposed transaction if it agrees either (i) to fund [**] percent ([**]%) of ongoing Development and commercialization costs for the applicable Development Candidate(s) or Product(s), or (ii) [**] or [**] and any related rights pursuant to [**]; and *provided further*, that failure of Foundation to communicate its approval or denial of a transaction pursuant to Second Amendment Section 10(e) shall entitle PTC to treat the proposed transaction as approved by Foundation. If the Foundation denies approval in accordance with this Second Amendment Section 10(e), Company shall not enter into such proposed definitive written agreement, but shall have the right to continue the applicable negotiations consistent with

this Second Amendment Section 10 for the purposes of achieving a form of such definitive written agreement acceptable to Foundation."

4. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Third Amendment and the terms of the Agreement, the First Amendment, or the Second Amendment, the terms of this Third Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Third Amendment.

IN WITNESS WHEREOF, the parties have executed this Third Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.		**SPINAL MUSCULAR ATROPHY FOUNDATION**	
/s/ Mark E. Boulding		/s/ Florence (Loren) Eng	
By:	Mark E. Boulding	By:	Florence (Loren) Eng
Title:	SVP & General Counsel	Title:	President

EXHIBIT TA-1

Corrective Plan

PTC and SMAF are proposing this amended Research Plan and related budget as a Corrective Plan to continue their existing collaboration directed towards the discovery of compounds with the potential to treat spinal muscular atrophy. [**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of six pages were omitted. [**]

Summary

[**].

Goal of the research proposal:

[**].

EXHIBIT TA-2

Budget for Corrective Plan

Employee Type	Jan-11	Feb-11	Mar-11	Apr-11	May-11	Jun-11	Cost	Total FTEs
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]	[**]	[**]	[**]	

External Spend (PTC)	Jan-11	Feb-11	Mar-11	Apr-11	May-11	Jun-11	Cost
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend (PGI)	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]

[**]	[**]	Cost Assumptions	
		Employee Type	FTE Rate
[**]	[**]	[**]	[**]
[**] [**]		[**]	[**]
[**] [**]		[**]	[**]
[**] [**]		[**]	[**]
[**] [**]		[**]	[**]
[**] [**]		[**]	[**]

EXHIBIT TA-3

DC Criteria

Activity	Goals	Assay Description	Notes

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of ten pages were omitted.

[**]

EXHIBIT TA-4

Foundation Partnering Team

Name	Relationship to Foundation
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Execution version

AMENDMENT No. 4 TO SPONSORED RESEARCH AGREEMENT

This Fourth Amendment ("Fourth Amendment") to the Sponsored Research Agreement is effective as of the 22 day of November, 2011 (the "Fourth Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007, by the Second Amendment on May 1, 2009 (the "Second Amendment"), and by the Third Amendment on January 1, 2011 (as so amended, the "Agreement");

WHEREAS, the parties desire to further amend the Agreement to extend the DC Timeline Goal and the Research Term; and

WHEREAS, the parties have been coordinating with respect to Collaboration Activities involving a proposed License and Collaboration Agreement (the "Proposed Roche Agreement") by and among F. Hoffmann-La Roche Ltd, a Swiss corporation with an office and place of business at Grenzacherstrasse 124, 4070 Basel, Switzerland ("Roche Basel") and Hoffmann-La Roche Inc., a New Jersey corporation with an office and place of business at 340 Kingsland Street, Nutley, New Jersey 07110, U.S.A. ("Roche Nutley"; Roche Basel and Roche Nutley together referred to as "Roche") on the first hand, the Company on the second hand and (solely with respect to the Foundation Provisions (as defined in the Proposed Roche Agreement)) the Foundation on the third hand, which Proposed Roche Agreement is expected to be finalized in the near future;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Fourth Amendment, the parties agree as follows:

1. Extension. The parties hereby agreed to extend the DC Timeline Goal to [**] and the Research Term until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) [**] or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3. If a Development Candidate is not selected by the extended DC Timeline Goal, the parties shall have the right to call a special meeting of the JSC to address [**] in accordance with Second Amendment Section [**], including by agreeing upon [**], and if applicable thereafter, the rights specified in Section Amendment Section [**] and/or Second Amendment Section [**].

2. SMAF Funding Amount. As of the Fourth Amendment Effective Date, the SMAF Funding Amount shall be $[**].

3. Proposed Roche Agreement. With respect to the Proposed Roche Agreement, the Company and the Foundation agree as follows: (a) the Foundation hereby waives the requirement for an in-person meeting set forth in Second Amendment Section 10(d)(iii) with respect to the Proposed Roche Agreement, (b) the Foundation approves the Proposed Roche

Agreement pursuant to Second Amendment Section 10(e), and (c) the effectiveness of this Fourth Amendment Section 3 shall be contingent upon the execution by the Company, Foundation and Roche of the definitive final version of the Proposed Roche Agreement.

4. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Fourth Amendment and the terms of the Agreement, the terms of this Fourth Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Fourth Amendment.

IN WITNESS WHEREOF, the parties have executed this Fourth Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.		**SPINAL MUSCULAR ATROPHY FOUNDATION**	
/s/ Stuart Peltz		/s/ Florence Eng (Loren)	
By:	Stuart Peltz	By:	Florence Eng (Loren)
Title:	President and CEO	Title:	President

Exhibit 10.16

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

DATED 26th of May 2010

(1) THE WELLCOME TRUST LIMITED

and

(2) PTC THERAPEUTICS, INC.

AGREEMENT FOR THE PROVISION OF FUNDING TO PTC THERAPEUTICS, INC. FOR RESEARCH RELATING TO SELECTIVELY DECREASING THE PRODUCTION OF BMI-1 EXPRESSION IN TUMOUR STEM CELLS

THIS AGREEMENT is made and entered into as of the 26th day of May 2010 (the “Commencement Date”)

BETWEEN:

(1) **THE WELLCOME TRUST LIMITED** a company registered in England under number 2711000 as Trustee of the Wellcome Trust, a charity registered in England under number 210183, whose registered office is at 215 Euston Road, London NW1 2BE (the “**Trust**”); and

(2) **PTC THERAPEUTICS, INC.** a company incorporated and registered in the State of Delaware whose principal place of business is at 100 Corporate Court, South Plainfield, NJ 07080-2449, USA (“**PTC**”).

RECITALS:

(A) PTC is undertaking a research programme aimed at identifying small molecules that selectively decrease the Production of Bmi-1 expression in tumour stem cells (“**Bmi-1**”);

(B) PTC is interested in receiving funding to enable the optimisation and early development of small molecules discovered under the research programme; and

(C) In order to further its charitable objectives, the Trust is willing to make a funding award (the "**Award**") to PTC under the Trust's Seeding Drug Discovery Strategic Award Programme to enable PTC to undertake the research programme set out in the Application (as defined below) in accordance with the provisions of this Agreement.

1. INTERPRETATION

1.1 In this Agreement, unless the context otherwise requires:

"Accounting Standard"	means in the case of PTC and its Affiliates, US GAAP (United States Generally Accepted Accounting Principles), and in the case of Trust and its Affiliates, IFRS (International Financial Reporting Standards), in either case as generally and consistently applied throughout each Party's organisation, provided, that PTC and its Affiliates may elect to convert to IFRS at any time on an organization-wide basis;
"Affiliate"	means, with respect to a given entity, any person, corporation, partnership or other entity, that Controls, is Controlled by, or is under common Control with such entity;
"Agreement"	means this agreement;
"Application"	means the application made by PTC to the Trust for an award as set out at Schedule 1 as amended by this Application;

"Auditor"	Shall have the meaning given to it in Clause 14;
"Award"	Shall have the meaning given to it in the Recitals;
"Award Amount"	means an award of up to five million and three hundred and ninety-seven thousand United States dollars (US$5,397,000);
"Background Intellectual Property"	means: (a) any Intellectual Property other than the GEMS Intellectual Property created, devised, generated, owned by or licensed to the PTC Group or which the PTC Group has rights to prior to the Commencement Date (but excluding, for the avoidance of doubt, the Programme Intellectual Property), which are necessary or useful for undertaking the Programme, for the protection or exploitation of the Programme Intellectual Property, and/or which are necessary or useful for the development and exploitation of the Programme Intellectual Property; and (b) The following patents or patent applications: [**];
"Background Know-How"	Means any and all Know-How and any components thereof (including without limitation manufacturing processes and quality control procedures) other than the GEMS Know-How which are used by the PTC Group at any time for the Programme which are necessary or useful for undertaking the Programme, for the protection or exploitation of the Programme Intellectual Property, and/or which are necessary or useful for the development and exploitation of the Programme Intellectual Property, but excluding the Programme Intellectual Property. For the avoidance of doubt, PTC Background Know-How shall include Know-How generated by any PTC Affiliate and subsequently used by PTC for the Programme;
"Base Shares"	shall have the meaning given to it in Schedule 6;
"Books"	shall have the meaning given to it in Clause 14;
"Business Day"	means a day on which banks are normally open for business and which is not a Saturday or Sunday or a bank or public holiday in England, or in the State of New Jersey;

"Change of Control"	Means the acquisition by any Third Party of Control of PTC, other than (a) acquisitions by employee benefit plans sponsored or maintained by PTC, (b) the initial public offering of PTC, (c) the acquisition by an institutional investor (or group of institutional investors), such as a venture capital fund, private equity fund or hedge fund, of shares of PTC for investment purposes in a transaction approved by PTC's Board of Directors, or (d) a business combination involving PTC pursuant to which the stockholders of PTC immediately prior to such business combination beneficially own directly or indirectly more than fifty percent (50%) of the then outstanding common shares or voting power of the entity resulting from such business combination;
"Clinical Trial"	means a clinical trial conducted in accordance with recognised protocols approved by a Competent Authority;
"Co-applicants"	means Dr. Young-Choon Moon and Dr. Marla Weetall of PTC;
"Commencement Date"	means the date of this Agreement as set out at the top of page 3;

"Competent Authority" means any local or national agency, authority, department, inspectorate, minister, ministry official or public or statutory person (whether autonomous or not) of, or of any government of, any country having jurisdiction over this Agreement or any of the Parties or over the development or marketing of drugs including the European Commission and the European Court of Justice, the US Food and Drug Administration, the European Medicines Agency (or any successor entity) and any national regulatory authorities;

"Conditions" means the conditions set out at Schedule 2 which must be satisfied (to the reasonable satisfaction of the Trust) at all times during the Programme;

"Confidential Information" means any and all data, results, Know-How, show how, software, plans, details of research work, discoveries, inventions, intended publications, intended or pending patent applications, designs, technical information, business plans, budgets and strategies, business or financial information or other information in any medium and in any form, and any physical items, prototypes, compounds, samples, components or other articles or Materials disclosed on or after the Commencement Date of this Agreement by one Party to another Party whether orally or in writing or in any other form including for the avoidance of doubt Background Intellectual

Property and Background Know-How;

"Control" means, in relation to PTC, where a person (or persons acting in concert) directly or indirectly including through any subsidiary or holding company or subsidiary of such holding company:

(a) has beneficial ownership over more than fifty per cent (50%) of the total voting rights conferred by all the issued shares in the capital of the company which are ordinarily exercisable in general meeting; or

(b) has the right to appoint or remove a majority of its directors; or

(c) has power to direct that the affairs of the company are conducted in accordance with its wishes;

in each case where such person or persons did not have such beneficial ownership, right or power at the Commencement Date;

and "**Controlled**" and "**Controlling**" shall be construed accordingly;

"Cost of Goods" means in respect of any Products the fully allocated cost of manufacture as calculated in accordance with the accounting standard applicable to the selling party, consistently applied in accordance with Schedule 4 together with any and all royalties payable to any Third Party for technology directly related to the supply of the Products including without limitation any formulation technology and access to Materials owned by Third Parties;

"Development" means research and development activities relating to the Programme Intellectual Property and any Products following completion of the Programme including Clinical Trials and "**Develop**" shall be construed accordingly;

"Disclosure Letter" means:

(a) as at the Commencement Date, the disclosure letter dated the same date as this Agreement and accepted by the Trust, and

(b) after the Commencement Date, the disclosure letter as subsequently amended and agreed by the Parties immediately prior to the payment of each Tranche (or first installment of the each Tranche) of the Award Amount;

"Distributor" means a Third Party with whom PTC enters into a standard commercial distribution or sales arrangement with respect to marketing or sales of Product in a particular territory or region. For clarity, although PTC may grant a licence to a Distributor in support of such arrangement, a Distributor shall not constitute a Licencee for the purposes of this Agreement.

"Documents" means reports, research notes, charts, graphs, comments, computations, analyses, recordings, photographs, paper, notebooks, books, files, ledgers, records, tapes, discs, diskettes, CD-ROMs, computer programs and documents thereof, computer information storage means, samples of material, other graphic or written data and any other media on which Know-How can be permanently stored;

"End of Award Report" shall have the meaning given to it in Clause 2.5;

"Expert" shall have the meaning given to it in Clause 19;

"Exploit" means exploitation activities after completion of the Programme including obtaining Marketing Approval and the commercialisation, licensing, marketing, distribution and sales of Programme Intellectual Property and any Products utilising the Programme Intellectual Property including on a Not-for-Profit Basis and "**Exploited**" and "**Exploitation**" shall be construed accordingly;

"Exploiting Party" means the Party or Parties undertaking development and exploitation pursuant to Clauses 11 and 12;

"Field" means the use of small molecules that selectively decrease the expression of Bmi-1 in tumour stem cells for the treatment, mitigation, diagnosis or prevention of disease in man or other animals;

"For Profit Basis" means sales of Products on other than a Not For Profit Basis;

"FTE" means one full time equivalent employee based upon a total of [**] hours worked per annum, not including time off; provided, that employees shall be accounted for on a percentage of effort basis;

"GEMS" means PTC's drug discovery technology for the modulation of gene expression by small molecules.

"GEMS Intellectual Property" means:

(a) any Intellectual Property created, devised,

generated, owned by or licensed to the PTC Group or to which the PTC Group has rights (but excluding, for the avoidance of doubt, the Programme Intellectual Property), which relates to the GEMS technology; and

(b) The following patents or patent applications:

[**]

"GEMS Know-How" Means any and all Know-How and any components thereof directly and solely relating to the GEMS technology.

"Intellectual Property" means:

(a) patents, designs, trade marks and trade names (whether registered or unregistered), copyright and related rights, database rights, Know-How and confidential information;

(b) all other Intellectual Property and similar or equivalent rights anywhere in the world which currently exist or are recognised in the future; and

(c) applications, extensions and renewals in relation to any such rights;

"Invention Policy" shall have the meaning given to it in Clause 4.3;

"IPMG" means the Intellectual Property Management Group established in accordance with Clause 9;

"IPMG Member" means a member of the IPMG;

"Know-How" means any technical and other information which is not in the public domain, including information comprising or relating to concepts, discoveries, data, designs, formulae, ideas, inventions, methods, models, assays, research plans, procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development), processes (including manufacturing processes, specifications and techniques), laboratory records, chemical, pharmacological, toxicological, clinical, analytical and quality control data, trial data, case report forms, data analyses, reports, manufacturing data or summaries

and information contained in submissions to and information from ethical committees and regulatory authorities and computer programs or algorithms. Know-How includes Documents containing Know-How, including but not limited to any rights including trade secrets, copyright, database or design rights protecting such Know-How. The fact that an item is known to the public shall not be taken to preclude the possibility that a compilation including the item, and/or a development relating to the item, is not known to the public;

"Lead Compound" means any Programme Compound which satisfies the criteria set out in Milestone 1 in Schedule 3 for use in the Field;

"Licencee" means a Third Party other than a Distributor to whom PTC grants a license to Exploit Programme Intellectual Property in the Field in a *bona fide*, arms-length transaction.

"Major Market" means any of the United States, the United Kingdom, Japan, and any two of the following: France, Spain, Germany and Italy.

"Marketing Approval" mean all approvals, licences, registrations or authorisations of any federal, state or local regulatory agency, department, bureau or other governmental entity, necessary for the marketing and sale of Products in a regulatory jurisdiction (including in the case of countries where no national agency exists for the approval of small molecules, approval by the World Health Organisation);

"Material" means any chemical or biological substance other than the GEMS assay and its component materials including any:

(a) organic or inorganic element;

(b) nucleotide or nucleotide sequence including DNA and RNA sequences;

(c) gene;

(d) vector or construct including plasmids, phages or viruses;

(e) host organism including bacteria, fungi, algae, protozoa and hybridomas;

(f) eukaryotic or prokaryotic cell line or expression system or any development strain or Product of that cell line or expression

system;

(g) protein including any peptide or amino acid sequence, enzyme, antibody or protein conferring targeting properties and any fragment of a protein or a peptide enzyme or antibody;

(h) drug or pro-drug;

(i) assay or reagent;

(j) any other genetic or biological material or micro-organism; data for the derivation of molecular structures including NMR spectra, X Ray diffraction patterns and other primary experimental information, assignments and other calculations required for determination of the structure, and co-ordinates of the derived molecular structure; and

(k) transgenic animals;

"Milestones" means the Milestones as described in Schedule 3, and "**Milestone**" means any one of them;

"Milestone Dates" means the dates set out in Schedule 3 for the achievement of a Milestone and "**Milestone Date**" means any one of them;

"Milestone Extension" shall have the meaning given to it in Clause 2.4;

"Milestone Report" shall have the meaning given to it in Clause 2.3;

"Net Sales" means the net sales on behalf of PTC and any of its Affiliates or Distributors for the Products sold to Third Parties other than Licensees, as determined in accordance with PTC's usual and customary accounting methods, which are in accordance with the Accounting Standards.

(a) In the case of any sale or other disposal of a Product between or among PTC and its Affiliates or Distributors, for resale, Net Sales shall be calculated only on the value charged or invoiced on the first arm's-length sale thereafter to a Third Party;

(b) In the case of any sale which is not invoiced or is delivered before invoice, Net Sales shall be calculated at the time of shipment or when the Product is paid for, if paid for before shipment or invoice;

(c) In the case of any sale or other disposal for value, such as barter or counter-trade, of any

Product, or part thereof, other than in an arm's length transaction exclusively for money, Net Sales shall be calculated on the value of the non-cash consideration received or the fair market price (if higher) of the Product in the country of sale or disposal; and

(d) In the event the Product is sold as a Combination Product, the Net Sales of the Product, for the purposes of determining royalty payments, shall be determined by multiplying the Net Sales of the Combination Product by the fraction, A/(A+B) where A is the weighted (by weight, if the Product is priced by weight; otherwise, by patient dose,) average sale price in a particular country of the Product when sold separately in finished form and B is the weighted average sale price in that country of the other product(s) sold separately in finished form. In the event that such average sale price cannot be determined for both the Product and the other product(s) in combination, PTC shall in good faith propose a relative allocation of value (and supporting methodology) for determining Net Sales for purposes of royalty payments with respect to such Combination Product, and Trust shall consider such proposal in good faith, and the Parties shall seek to reach agreement on such allocation. If the Parties are unable to reach such agreement within [**] days after PTC provides such proposal, the issue shall be referred for binding resolution to a mutually agreeable individual (not affiliated with either Party) with expertise in the marketing and sales of similar

pharmaceutical products similar to the Combination Product at issue (including experience in pricing and reimbursement), such resolution to occur within [**] Business Days after such referral.

"Non-Exploiting Party" means the Party or Parties not undertaking Development and Exploitation;

"Not for Profit Basis" means (a) sales of Products where the consideration received by the seller is less than or equal to the sum of the Cost of Goods for such Product; in calculating the consideration received by the seller account shall be taken of any equity or lump sum or other payments received by the seller in respect of the Product; or (b) or sales of Products where the seller is a charitable organisation (other than the Trust or its

Affiliates) under applicable law;

"Parties" means the parties to this Agreement, or any of them, as the context may require, and "**Party**" shall be interpreted accordingly;

"Phase 1 Clinical Trial " means a human clinical trial in any country, the principal purpose of which is a preliminary determination of safety in individuals or patients, that would satisfy the requirements of 21 C.F.R. §312.21(a), or an equivalent clinical study required by a Competent Authority outside the United States.

"Phase 2 Clinical Trial " means a human clinical trial conducted in any country, intended to explore multiple doses, dose response or duration of effect to generate initial evidence of safety and activity in a target patient population, that would satisfy the requirements of 21 C.F.R. §312.21(b), or an equivalent clinical study required by a Competent Authority outside the United States.

"Phase 3 Clinical Trial " means a human clinical trial in any country that would satisfy the requirements of 21 C.F.R. §312.21(c), or an equivalent clinical study required by a Competent Authority outside the United States that is prospectively designed to confirm with statistical significance in an expanded patient population the efficacy and safety of a drug in a given patient population, and the results of which are intended, alone or in combination with any other Clinical Trial, to form the basis for Marketing Approval by a Competent Authority.

"PTC Group" means PTC and any Affiliate of PTC;

"Policies and Positions" means the policies and positions of the Trust for grants from time to time, which are set out at http://www.wellcome.ac.uk/node3610.html;

"Principal Investigator" means Dr. Thomas Davis of PTC;

"Product" means any Product developed by any member of the PTC Group or any Third Party incorporating, comprising or derived from the Programme Intellectual Property in finished dosage pharmaceutical form;

"Programme" means the research and development programme described in the Application and funded by the Trust pursuant to the Award and the terms of this Agreement; provided, that the RSG may amend the research and development programme from time to

time in accordance with this Agreement;

"Programme Compound" means any compound identified or designed by PTC or another member of the PTC Group based on any hit or hits against the Bmi-1 target identified following screening and in respect of which activities are undertaken by (or on behalf of) PTC in the course of the Programme, and in each case shall include the chemical compound as well as esters, salts, hydrates, solvates, polymorphs and isomers thereof;

"Programme Books and Records" shall have the meaning given to it in Clause 4.4;

"Programme Intellectual Property" means any Intellectual Property (including the Programme Patents) created, devised or arising out of PTC or Staff undertaking and performance of the Programme or any part of it, including, without limitation, the Lead Compounds;

"Programme Inventions" means any inventions created, devised or arising out of PTC or Staff undertaking and performance of the Programme or any part of it;

"Programme Patents" means any patent applications that may be made by a member of the PTC Group or by the Trust on behalf of a member of the PTC Group (as appropriate) which claim any Programme Inventions or parts thereof, and any patents resulting from any such applications, utility certificates, improvement patents and models and certificates of addition and all foreign counterparts of them in all countries, including any divisional applications and patents, refiling, renewals, continuations, continuations-in-part, patents of addition, extensions (including patent term extensions), reissues, substitutions, confirmations, registrations, re validations, pipeline and administrative protections and additions, and any equivalents of the foregoing in any and all countries of or to any of them, as well as any supplementary protection certificates and equivalent protection rights in respect of any of them;

"Programme Term"	means the time period commencing on the Commencement Date and ending on the earlier of completion of the Programme or three (3) years.
"PubMed Central"	means an archive of life science journal literature operated by the National Center for Biotechnology Information, a division of the US National Library of Medicine accessible at http://www.pubmedcentral.nih.gov/;
"Quarter"	means a period of three (3) consecutive calendar months commencing on 1 January, 1 April, 1 July or

	1 October in any year and " **Quarterly**" shall be construed accordingly;
"Research Steering Committee" and "RSG"	means the group of persons constituted in accordance with Clause 5;
"Revenue Sharing Terms"	shall have the meaning given to it in Schedule 6;
"Site Visit Group"	means the group constituted in accordance with Clause 6;
"Staff"	means all scientific and technical staff, who are employees, students, officers, contractors, independent consultants or otherwise of PTC (or any other member of the PTC Group) and who participate in the Programme including without limitation the Principal Investigator and the Co-applicants, together with any relevant administrative staff assisting with the Programme;
"Third Party"	means any entity or person other than the Parties or an Affiliate of a Party;
"Tranches"	means the tranches of the Award Amount payable by the Trust to PTC as set out in Schedule 3, and "**Tranche**" shall mean each of them;
"Trust Contribution"	Means (a) tranches of the Award Amount paid by the Trust to PTC and (b) in the event a Milestone has been completed but PTC (i) has failed to submit a Milestone Report or request payment of the next tranche of the Award Amount, or (ii) is otherwise in breach of the Agreement and such breach would give rise to a termination right on the part of the Trust pursuant to Clause 20.2 or 20.3, those tranches of the Award Amount that would have been payable by the Trust to PTC but for PTC's omission or breach;
"Valid Claim"	shall mean a claim of an issued Patent Right of the Programme Intellectual Property, or a claim of a pending patent application or a supplementary protection certificate of a Patent Right of the Programme Intellectual Property that has not expired or been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment; provided however that such claim within a patent application has not been revoked, cancelled, withdrawn, held invalid or abandoned or been pending for more than [**] years from the date of its first priority filing anywhere in the world;

"Value Added Tax"	shall have the meaning given to it in Clause 13.6; and
"Warranties"	means the warranties given by PTC to the Trust as set out in Clause 18.2.

1.2 References in this Agreement to any statutory provisions shall be construed as references to those provisions as respectively amended consolidated or re enacted (whether before or after the Commencement Date) from time to time and shall include any provisions of which they are consolidations or reenactments (whether with or without amendment).

1.3 Reference to any statute, statutory instrument, regulation, by law or other requirement of English law and to any English legal term for any actions, remedy, method of judicial proceeding, legal document, legal status, court, official or any legal concept or doctrine shall, in respect of any jurisdiction other than England, be deemed to include that which most nearly approximates in that jurisdiction to the relevant English term.

1.4 The Schedules and Recitals form part of this Agreement and any reference to this Agreement shall include the Schedules and Recitals.

1.5 In this Agreement:

(a) the masculine gender shall include the feminine and neuter and the singular number shall include the plural and vice versa;

(b) references to persons shall include bodies corporate, unincorporated associations, partnerships and individuals;

(c) except where the contrary is stated, any reference in this Agreement to a Clause or Schedule is to a Clause of or Schedule to this Agreement, and any reference within a Clause or Schedule to a sub Clause, paragraph or other sub-division is a reference to such sub Clause, paragraph or other sub-division so numbered or lettered in that Clause or Schedule.

1.6 The headings in this Agreement are inserted for convenience only and shall not affect the construction of the provision to which they relate.

1.7 References to the winding-up of a person include the amalgamation, reconstruction, reorganisation, administration, dissolution, liquidation, bankruptcy, merger or consolidation of such person and an equivalent or analogous procedure under the law of any jurisdiction in which that person is incorporated, domiciled or resident or carries on business or has assets.

1.8 Any reference to books, records or other information includes books, records or other information in any format or medium including paper, electronically stored data, video or audio recordings and microfilm.

1.9 Any phrase introduced by the terms “including”, “include”, “in particular” or any similar expression shall be construed as illustrative and shall not limit the sense of the words preceding those terms

1.10 Where reference is made in this Agreement to the prior written consent of the Trust being required in respect of any matter, the Company shall give not less than [**] Business Days notice to the Trust of the matter for which such consent is required.

2. **AWARD**

2.1 The Award Amount will be payable in Tranches as set out in Schedule 3. Any Tranche may be paid in smaller instalments as may be determined by the Trust.

2.2 The Trust shall pay the first instalment of the first Tranche of the Award Amount to PTC within [**] Business Days of the Commencement Date. PTC undertakes to commence the Programme within [**] months of receipt of the first instalment of the first Tranche of the Award Amount.

2.3 When PTC considers that any Milestone has been achieved by the relevant Milestone Date:

(a) PTC shall as soon as reasonably practicable provide the Trust with a detailed report (the “**Milestone Report**”) setting out how the Milestone was achieved and requesting payment of the next Tranche of the Award Amount; and

(b) The Trust shall confirm to PTC in writing, within [**] Business Days of receipt by the Trust of the Milestone Report either that:

(i) the Milestone has been achieved by the Milestone Date to the Trust’s reasonable satisfaction, in which case the Trust shall make payment of the next Tranche of the Award Amount within [**] Business Days of the date of such written confirmation in the amount determined by the Trust from time to time; or

(ii) the Milestone has not been achieved to the Trust’s reasonable satisfaction by the relevant Milestone Date and that the payment shall not take place, in which case the Trust shall provide PTC with reasonable details of the grounds on which it has reached this decision.

2.4 The Trust may, at its sole discretion, grant PTC a reasonable period of time (“**Milestone Extension**”), in order to address the reasons why the Trust has judged that a particular Milestone has not been met. Upon the expiry of a Milestone Extension, the Trust shall, at its sole discretion, decide whether or not to permit full or partial payment of the relevant Tranche of funding to PTC.

2.5 PTC shall complete and submit a detailed report on the work done and outcomes of the Programme (“**End of Award Report**”) in the prescribed form to the Trust, such report to be presented to the Trust within [**] days after the completion of the Programme (or such other date as may be agreed with the Trust). The Trust will evaluate the End of Award Report and will notify PTC within [**] Business Days of receipt whether the report is acceptable to the Trust. If the End of Award Report is not acceptable to the Trust, it shall notify PTC of its reasons at the same time, which may include that the report is incomplete or insufficiently detailed and the Trust shall have

the right to withhold further funding until the Trust receives an End of Award Report which the Trust deems to be acceptable.

2.6 The Trust will only be obliged to pay any Tranche to PTC if, at the time of request for payment from PTC or the due date for payment of the Tranche:

(a) none of the events described in Clauses 20.2, 20.3, 20.4(a), or 20.5 have occurred or would result from the proposed payment;

(b) PTC is not in breach of any of any of the Conditions;

(c) the Warranties are true and correct in all respects, subject to the matters set out in the relevant Disclosure Letter;

(d) the Trust has received the relevant Disclosure Letter and the contents of such Disclosure Letter are reasonably acceptable to the Trust; and/or

(e) the Site Visit Group has conducted a review of PTC’s facilities in accordance with Clause 6 and such visit has not been completed to the reasonable satisfaction of the Trust.

2.7 If any Milestones have not been achieved by [**] years from the Commencement Date, unless agreed otherwise in writing by the Trust, the Trust shall have no obligation to pay any Tranche (or part thereof) which has not been paid prior to that date.

2.8 All payments made by the Trust to PTC under this Agreement shall be made in United States dollars ($). PTC shall ensure that it holds a bank account in the currency in which the Award Amount shall be advanced. Payment shall be made by electronic wire transfer of immediately available funds directly to PTC’s account designated below or to such other account as PTC may specify by written notice.

Bank Account for PTC:

Account Name: PTC Therapeutics, Inc.

Account No.: [**]

ABA No.: 031201467

Swift No.: PNBPUS33

Bank: Wachovia Bank NA

Branch address: MAC N 2684-020, 120 Mountain View Blvd., Suite 200, Basking Ridge, NJ 07920, USA.

2.9 The Trust shall not be under any obligation to pay any part of the Award Amount to PTC unless PTC operates a treasury policy that is approved by the Trust.

2.10 Each of the Trust and PTC shall (and PTC shall procure that relevant members of the PTC Group shall) pay any and all taxes levied in respect of all payments it receives or makes under this Agreement. Any withholding or other taxes that is required by law to be withheld or paid, with respect to any payments to it under this Agreement, shall be deducted from such payments and paid contemporaneously with the remittance, together with evidence of such withholding or payment. Such withholding and payment shall fully discharge the party making the payment and no further payment shall be required by the payor to the payee. The party withholding or making such payment shall furnish the other party with appropriate documents to secure application of the most favourable rate of withholding tax under applicable law.

2.11 PTC undertakes to use all funding received from the Trust pursuant to this Agreement solely for the purposes of the Programme as described in the Application. PTC shall obtain the Trust's prior written consent to any other use of any funding received from the Trust pursuant to this Agreement.

2.12 Subject to Clause 2.13 below, PTC undertakes that it shall not (and PTC shall procure that other members of the PTC Group shall not) seek to apply for or accept without the Trust's prior written consent (such consent not to be unreasonably withheld) any other funding or support (whether in kind or otherwise) for the programme of research agreed for the Programme, whether commercial or non-commercial, during the period of the Programme.

2.13 PTC hereby grant to the Trust a first option to consider any further requirements of PTC for the funding of the programme of research agreed for the Programme or any further development of the results of the Programme for a period of [**] years following the end of the Programme Term. Any such further funding requirements shall be notified to the Trust by PTC (as the case may be), and the Trust shall within [**] days of such notification indicate to PTC whether the Trust wishes to so further fund (in whole or in part), subject to the proper Trust funding application and review process being carried out. If the Trust does not so elect, then the option shall lapse. For clarity, this Clause 2.13 is not intended to restrict PTC from engaging in general capital raising activities provided that such funds are not specifically earmarked for the Programme.

3. THE PROGRAMME

3.1 PTC undertakes to use its reasonable endeavours to achieve each Milestone on or before each relevant Milestone Date.

3.2 PTC undertakes to diligently perform the research and the Programme management of the Programme, as set out in the Application and as determined by the RSG and the IPMG from time to time.

3.3 Subject to existing confidentiality obligations and legal restrictions, including contractual restrictions, PTC shall inform the RSG in writing of any on-going research being carried on by PTC or any on-going research that, to the knowledge of PTC, is being carried on by any other member of the PTC Group in the Field. PTC undertakes, throughout the duration of this Agreement, to use its reasonable endeavours to co-operate with and to adopt

a synergistic and collaborative approach to work with Third Parties working on similar research and development programmes to the Programme.

4. PROGRAMME MANAGEMENT AND PROGRAMME AUDIT

4.1 PTC shall appoint a Programme manager from its Staff who shall be responsible on a day-to-day basis for co-ordinating the internal and external components of the Programme.

4.2 PTC warrants that it has or that it shall have in place contracts with its Staff such that any Programme Intellectual Property shall be owned by and assigned to PTC and that each member of the Staff is obliged to waive (to the extent that such rights exist under applicable law and are waivable) all moral rights and rights of a like nature in the Programme Intellectual Property. The Trust may upon reasonable notice require PTC to produce all and any Staff contracts for inspection by the Trust except as may be limited by applicable laws.

4.3 PTC shall cause to be kept full, detailed and accurate records of all of its activities and results obtained in connection with the Programme. In this respect, PTC shall and shall procure that the Staff shall at all times:

(a) observe professional standards; and

(b) maintain a policy which requires its Staff or others acting on its behalf to record and maintain all data and information developed during the Programme in such a manner as to enable the Parties to use such records to establish the earliest date of invention and/or diligence to reduction to practice (an "**Invention Policy**"). Such Invention Policy shall, among other things, provide that such individuals are to keep record all research, development and other work carried out in respect of the Programme and the results of such research, development and other work, in standard laboratory notebooks (or electronic equivalents that meet the requirements of applicable law) that are dated and corroborated by non-inventors on a regular, contemporaneous basis.

4.4 PTC shall maintain, or cause to be maintained, books and records of its activities under the Programme and its expenditure of the Trust Award (the "**Programme Books and Records**") in sufficient detail and in good scientific manner appropriate for audit, patent and regulatory purposes, which shall be complete and accurate and shall fully and properly reflect all work done and results achieved in the performance of its respective activities under the Programme, and which shall be retained by PTC for at least [**] years after the creation of the record, or for such longer time period prescribed by PTC's document retention policies or as may be required by applicable law. The Trust shall have the right, during normal business hours and upon reasonable

notice, to inspect and copy any Programme Books and Records if required for audit, patent or regulatory purposes provided that the Trust shall not be entitled to exercise this right more than [**] in any calendar year, shall only use such information for the purposes of exercising its rights or complying with its obligations under this Agreement, and shall treat such Programme Books and Records and any copies thereof as Confidential Information.

4.5 PTC shall procure that the control of expenditure to be funded under the Award is governed by the normal accounting standards and procedures applicable to members of the PTC Group and such expenditure shall be covered by the formal audit arrangements that exist within the PTC Group. Following the annual audit of PTC by its external auditors, PTC shall provide the Trust with the audited financials which shall indicate whether the external auditors have signed their opinion on the annual accounts of PTC without qualification. PTC shall further confirm in writing to the Trust that the management letter from the auditors raises no matters that did or could significantly affect the administration of grants awarded by the Trust. If the auditors have raised any such matters in their management letter, PTC shall, on request from the Trust, provide the Trust with relevant extracts from the letter.

4.6 During the Programme Term and for a period of [**] years afterwards, the Trust shall have the right, at its discretion and expense, to audit (either directly or via a Third Party engaged by it):

(a) any expenditure of the Award Amount including, without limitation, any expenditure by PTC, any other member of the PTC Group, Co-applicants, collaborators and/or subcontractors;

(b) the systems used by PTC to administer Trust grants generally; and

(c) any equipment acquired under the Award Amount.

Provided, that the Trust shall not be entitled to exercise this audit right more than [**] in any calendar year, and shall only use such information for the purposes of exercising its rights or complying with its obligations under this Agreement, and shall treat the any documents reviewed or information received as a result of such audit as Confidential Information.

4.7 In furtherance of the Trust's audit right pursuant to Clause 4.6, PTC shall (and shall procure that its Affiliates shall) provide access at any time during business hours for auditors and other personnel from or appointed by the Trust to accounting and other financial and corporate records relating to this Agreement, the Award, the Programme Books and Records, and the activities funded by the Trust (at the Trust's expense), if requested at any time on reasonable advance notice. Where any of the Award Amount has been paid by PTC to any collaborators and/or sub-contractors, PTC shall use its best efforts to procure that the right of access for audit purposes extends to the accounts and records of any such collaborator and subcontractor.

4.8 PTC shall maintain a separate accounting cost code specific to the Award, and all costs and income properly relating to the Award shall be accounted for through that cost code. PTC shall ensure that appropriate records are kept to support the entries made on the cost code.

4.9 PTC shall be responsible for the management, monitoring and control of all research work undertaken by it. This shall include, as appropriate, the requirements of all applicable laws and regulatory authorities, including but not limited to those governing the use of radioactive isotopes, diagnostic tools, animals, pathogenic organisms genetically modified organisms, toxic and hazardous substances, research on human subjects and human embryos, and include appropriate ethical approvals and consents, including

for example but not limited to, such approvals and consents for obtaining tissues and other human samples. For any clinical trial carried out pursuant to the Programme, PTC shall on the Trust's written request supply details of such clinical trial for publication on the Trust's clinical trials register and any applicable national clinical trials register.

4.10 Any research under the Programme that involves animals that is undertaken by any member of the PTC Group, collaborators, subcontractors or service providers (whether in the UK, United States or any other country) shall comply with both the Trust's policy on the use of animals in research and the principles set out in the document "Responsibility in the use of animals in bioscience research: Expectations of the major research council and charitable funding bodies" (http://www.wellcome.ac.uk/About-us/Policy/Policy-and-position-statements/WTD040129.htm). If procedures taking place in the UK and regulated under the UK Animals (Scientific Procedures) Act 1986 will be used, the research shall comply with such Act, be approved by the local ethical review process and be conducted with due consideration for the 3Rs (replacement, reduction and refinement of the use of animals in research). If procedures taking place in the US and regulated under the US Animal Welfare Act of 1966, as amended, the research shall comply with such Act.

4.11 Any research under the Programme that is undertaken by any member of the PTC Group, collaborators, subcontractors or service providers (whether in the UK, the United States or any other country) shall:

(a) comply with the World Medical Association's "Declaration of Helsinki - Ethical Principles for Medical Research Involving Human Subjects 2008" as amended from time to time;

(b) be subject to appropriate ethical review procedures in accordance with applicable law;

(c) comply with all applicable local legislation; and

(d) be approved by the local ethical review process.

5. THE RESEARCH STEERING GROUP

5.1 As soon as practicable following the Commencement Date, the Trust and PTC shall establish a Research Steering Group ("**RSG**") to oversee the Programme, which shall:

(a) monitor the performance and technical content of the Programme against the description outlined in the Application;

(b) assess the ongoing results of the Programme and what has been learnt and agree future research;

(c) critically assess the results of the Programme;

(d) identify and address any weaknesses or delays in the Programme;

(e) co-ordinate internal and outsourced components of the Programme, including agreeing on whether to pursue any collaborations or subcontracts not specifically identified in the Application;

(f) modify or authorise modifications to the implementation of the Programme (including the implementation of the Programme objectives) as necessary from time to time;

(g) operate as the key forum through which the Trust shall be informed as to progress of the Programme and through which the Trust shall liaise with PTC concerning the conduct of the Programme, including preparing an annual written report for the Trust on progress;

(h) advise the Trust when and whether each of the research phases, Milestones or targets of the Programme have been achieved; and

(i) review all proposed public disclosures relating to the Programme, including proposed presentations, posters and papers (ensuring that the contribution of the Trust is acknowledged in all such proposed publications and that the Trust's Award number is quoted) and advise the Trust as to the RSG's reasonable recommendations in respect of such proposed publications;

provided that the RSG shall have no right to amend or vary the terms of this Agreement or to alter the fundamental scope or objectives of the Programme which power is reserved to the Parties.

5.2 The RSG shall be established and run by the Parties as follows:

(a) The RSG shall comprise the following persons ("**Members**"):

(i) [**] representatives of PTC, one of whom shall be the Principal Investigator;

(ii) at least one independent industry adviser with experience which is relevant to the Programme;

(iii) [**] representatives or nominees of the Trust's Technology Transfer Division (at the Trust's option).

(b) The Trust shall have the option to appoint up to [**] Members and up to [**] observers to the RSG, to remove any Member or observer appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such Member or observer. In the event that the Trust does not appoint any Member or observer, the Trust shall have the right to receive all papers that a Member or observer would be entitled to receive.

(c) PTC shall have the option to appoint up to [**] Members and up to [**] observers to the RSG, to remove any Member or observer appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such Member or observer; provided, that the Principal Investigator shall always be one of PTC's Members.

(d) PTC and the Trust shall jointly agree the identity of the Members of the RSG who are independent industry adviser(s). The costs and expenses of the independent industry adviser(s) shall be met out of the Award Amount.

(e) The Principal Investigator shall be the chairperson of the RSG ("**RSG Chair**") and shall be responsible for organising meetings of the RSG, including preparing papers prior to meetings and ensuring that minutes of meetings are produced promptly after each meeting. All papers and minutes shall be circulated to each Member in a timely manner. Except in exceptional circumstances (when the Principal Investigator may nominate another person as his alternate), the Principal Investigator shall attend all RSG meetings.

(f) The quorum for RSG meetings shall be [**] Members including a [**]. Decisions of the RSG shall be made by majority agreement with each Party entitled to cast one (1) vote regardless of the number of Members present. If the RSG is unable to reach agreement on a decision, the decision shall be escalated to the Director of Technology Transfer at the Trust and the head of Discovery Research at PTC for resolution. For the avoidance of doubt, any observers appointed by the Parties shall not be Members of the RSG and shall not have a right to participate in its decision-making process unless otherwise agreed in writing by PTC and the Trust.

5.3 Meetings of the RSG shall be convened by the Principal Investigator as required but at least [**] months (or less frequently with the consent of the Trust) for the duration of the Programme Term, on not less than [**] Business Days' written notice (to be accompanied by an agenda for the meeting). Following the end of the Programme Term, the RSG shall meet within [**] Business Days to discuss and report on the outcomes of the Programme, and shall thereafter be dissolved.

5.4 Any or all Members may, with the prior consent of the RSG Chair, attend a meeting of the RSG by telephone or other electronic means rather than in person, provided that all Members attending the meeting can hear and be heard for all parts of the meeting. For the avoidance of doubt, RSG Members attending a meeting by telephone or other electronic means shall have the same voting rights as an RSG Member present in person.

5.5 A representative from any key outsourcing suppliers, collaborators or subcontractors involved in the Programme (if any) shall be invited to RSG meetings as an observer. The RSG shall also have power to invite persons whose special skills or influence might advance the Programme to attend and address

meetings of the RSG. Such persons shall not be Members of the RSG and shall not have a right to participate in its decision-making process. The RSG Chair shall ensure that any such invitees sign confidentiality agreements in a form acceptable to all parties.

5.6 PTC shall upon request make available to the Trust and/or the RSG copies of all records generated in connection with the Programme, including for the avoidance of doubt, records generated by its Staff under Clause 4.3 and by any Third Party collaborators to the Programme appointed under Clause 7.

5.7 During the Programme Term, PTC shall procure that the Principal Investigator monitors the work carried out under the Programme for material that may be the subject of Programme Inventions and shall promptly notify the RSG of any such Programme Invention. Without prejudice thereto, during the Programme Term, PTC shall make reports on work being carried out under the Programme to the RSG [**], or from time to time as the RSG may reasonably request, such reports to include the progress of the Programme as well the matters described in Clause 11.9.

6. SITE VISIT GROUP

6.1 The Trust may appoint a Site Visit Group, made up of a small team of independent experts and observers from the Trust's Technology Transfer Division. The Site Visit Group shall have reasonable access for the duration of the Programme during normal working hours and at mutually agreed times to visit all the premises where the Programme is being conducted to consult informally with PTC's employees, researchers, consultants or contractors working on the Programme to evaluate progress, performance and key issues and to report back to the Trust and the RSG on their findings.

6.2 The Site Visit Group may recommend that the Trust terminates the Programme due to a serious failure in the progress, management or conduct of the Programme (including but not limited to a finding that the Programme will be unable to achieve the next Milestone within a reasonable time period after the relevant Milestone Date), or due to a major external scientific, technical or commercial barrier which means that the Programme is unlikely to succeed in its objectives. If the Site Visit Group makes such a recommendation pursuant to this Clause 6.2, it must provide written notice of its recommendation and the rationale therefor to the Parties.

6.3 If PTC is unable to remedy a serious failure or external barrier identified by the Site Visit Group pursuant to Clause 6.2 within [**] Business Days, or such longer time period as the Trust may, in its sole discretion, allow, the Trust may terminate this agreement pursuant to Clause 20.3(b).

7. PROGRAMME COLLABORATORS AND SUBCONTRACTORS

7.1 If PTC wishes to use a Third Party collaborator or sub-contractor to conduct any part of the Programme, it shall seek the consent of the RSG unless such sub-contractor or collaborator is specified in the Application. PTC shall provide a copy of the agreed form of any sub-contract or collaboration agreement to the Trust for review by the Trust prior to signature by the parties thereto. Unless otherwise agreed in writing between the Parties, PTC shall ensure in all cases that any collaborations or sub-contracts shall be on the following terms:

(a) that the Third Party shall not have any rights to any results emerging from such work and all such results shall as between the Parties and the Third Party be deemed to be Programme Intellectual Property and owned in accordance with the provisions of this Agreement; provided, however, that if applicable law prevents assignment of ownership, PTC shall use its best efforts to secure appropriate license or option rights to such intellectual property;

(b) that the Third Party shall be under obligations of confidence concerning such results on terms equivalent to those set out under this Agreement;

(c) that the Third Party shall keep detailed records including scientific notebooks of all of its activities and upon request shall make available copies to the Trust, except where prohibited by applicable law;

(d) that the Third Party will upon reasonable request make available its premises where the Programme is being conducted, and its employees and/or consultants for discussion with the Site Visit Group as referred to in Clause 6, except where prohibited by applicable law; and

(e) that the provisions of such sub-contract or collaboration agreement shall be consistent with the nature of the Award and the payment of the Award Amount in Tranches, the Trust's rights pursuant to Clauses 12 and 13 and the termination provisions of this Agreement, and shall terminate if this Agreement terminates.

8. INTELLECTUAL PROPERTY — OWNERSHIP AND PROTECTION

8.1 In the event that any Programme Intellectual Property arises, it shall be the property of PTC. Any Programme Patents arising from such Programme Intellectual Property shall be applied for in the name of PTC. PTC shall have the first option to take responsibility for seeking and maintaining protection for Programme Intellectual Property in consultation with the RSG at PTC's sole cost, including the filing, conduct, prosecution and maintenance of all patents arising in respect of Programme Inventions.

8.2 If PTC chooses not to pursue filing, prosecution or maintenance of any Programme Patents in any country, it shall immediately notify the Trust of this fact in writing. The Trust shall be entitled, but not obliged, at its own cost, to pursue or maintain such Programme Patents in the relevant country or countries in PTC's name and PTC shall provide such assistance to the Trust at the Trust's sole cost as may reasonably be required by the Trust in order to do so.

8.3 Without prejudice to the terms of this Clause 8.3, PTC shall (and shall procure that the Principal Investigator shall) execute such further documents, take such action and do such things as may be reasonably requested by the Trust at the Trust's cost to secure the right of the Trust to protect, maintain, manage, defend, enforce and exploit the Programme Intellectual Property referred to in this Clause 8 and Clauses 9, 10, 11 and 12 below.

8.4 PTC shall make the Background Intellectual Property available for use in the Programme and for the protection, development and exploitation of the Programme Intellectual Property. PTC shall, unless otherwise agreed, retain responsibility for seeking and maintaining protection for the Background Intellectual Property at its own cost. If PTC chooses not to pursue filing, prosecution, maintenance, defence or enforcement of any patent rights that are

Background Intellectual Property in any country, they shall give the Trust at least [**] months' notice of this fact in writing. During the [**]-month notice period, PTC shall continue to seek and maintain such patent rights. The Trust shall be entitled, but not obliged, at its own cost, to assume

responsibility (on behalf of PTC) for filing, prosecuting, maintaining, defending or enforcing such patent rights in the relevant country or countries in PTC's name and PTC shall provide such assistance to the Trust at the Trust's cost as may reasonably be required by the Trust in order to do so.

9. **INTELLECTUAL PROPERTY MANAGEMENT GROUP**

9.1 PTC and the Trust shall establish an Intellectual Property Management Group ("**IPMG**"), which shall:

(a) approve all public disclosures relating to the Programme, including presentations, posters and papers (provided that the contribution of the Trust is acknowledged in all such publications and quoting the Award number);

(b) identify new inventions arising out of the Programme and make recommendations for IP strategy, including patent filing and prosecution strategy and assessment of freedom to operate issues; and

(c) approve the Exploiting Party's Development and Exploitation strategy in relation to the Programme Intellectual Property.

9.2 The IPMG shall be established and run by the Parties as set out below:

9.3 The IPMG shall be comprised of the following persons ("**IPMG Members**"):

(a) not more than [**] representatives of PTC;

(b) not more than [**] representatives of the Trust's Technology Transfer Division or their nominees.

9.4 The Trust shall have the option to appoint [**] IPMG Members, to remove any IPMG Member appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such IPMG Member. In the event that the Trust does not appoint such IPMG Members, the Trust shall have the right to receive all papers that an IPMG Member would be entitled to receive.

9.5 PTC shall have the option to appoint [**] IPMG Members, to remove any IPMG Member appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such IPMG Member. If PTC does not appoint such IPMG Members, PTC shall have the right to receive all papers that an IPMG Member would be entitled to receive.

9.6 The IPMG Members shall select a chair ("**IPMG Chair**") and the IPMG Chair shall be responsible for organising meetings of the IPMG, including preparing papers prior to meetings and ensuring minutes of meetings are produced. All papers and minutes shall be circulated to each IPMG Member in a timely manner.

9.7 The quorum for IPMG meetings shall be [**] IPMG Members, provided that at least [**]. Decisions of the IPMG shall be made by majority agreement with each Party entitled to case one (1) vote regardless of the number of IPMG Members present. If the IPMG is unable to

reach agreement on a decision, the decision shall be escalated to Director of Technology Transfer at the Trust and the General Counsel of PTC for resolution. If the IPMG Chair is unable to attend an IPMG meeting, PTC and the Trust shall, in good time before the meeting, nominate an alternative IPMG member to act as Chair.

9.8 Meetings of the IPMG shall be convened by the IPMG Chair at least [**] per year and otherwise on an "as needed" basis, either in person at the premises of PTC or by 'virtual private network' videoconference if necessary. Any or all IPMG Members may, with the prior consent of the IPMG Chair, attend a meeting of the IPMG by telephone or other electronic means rather than in person, provided that all IPMG Members attending the meeting can hear and be heard for all parts of the meeting. For the avoidance of doubt, IPMG Members attending a meeting by telephone or other electronic means shall have the same voting rights as an IPMG Member present in person.

10. **INFRINGEMENT**

10.1 Each Party shall immediately give notice to the other Party if any member of the Trust, the PTC Group or their Staff become aware of:

(a) any infringement of the Background Intellectual Property or Programme Intellectual Property; or

(b) any claim by a Third Party that an action carried out under the Programme infringes the Intellectual Property or other rights of any Third Party.

10.2 In respect of any Background Intellectual Property or Programme Intellectual Property, where any infringement or suspected infringement arises, or a claim by a Third Party alleging infringement of that Third Party's Intellectual Property or other rights arises, then:

(a) As soon as possible after receiving the notice required by Clause 10.1, the Parties will convene a meeting of the IPMG at which the IPMG shall discuss in good faith all available evidence with respect to the matters underlying the notice, and the appropriate manner of addressing such matters, including preventing or stopping infringing activities (for example, by seeking a preliminary injunction), preserving the Parties' rights to past and future damages (for example, by sending a cease and desist letter) defending against declaratory judgment actions with respect thereto, or taking any other actions, or no actions, as the Parties shall determine. The IPMG shall take into account each Party's interest in formulating the response, if any, to infringement or threatened infringement of such Background Intellectual Property or Programme Intellectual Property, including the relative merits of patent litigation versus the nature, scope and potential economic consequences of the Infringement.

(b) Unless otherwise determined by the IPMG as part of its consideration of an overall patent strategy, or pursuant to its evaluation of a notice pursuant to Clause 10.2(a), if a member of the PTC Group (or any licensee of a member of the PTC Group) is exploiting the relevant Programme Intellectual Property but PTC notifies the Trust that it does not intend to take such action, the Trust, at its discretion and cost may

take such action as it shall consider to be necessary or appropriate to bring or defend an action on behalf of the relevant member of the PTC Group or licensee thereof and PTC shall (and shall procure that relevant members of the PTC Group shall) provide all reasonable assistance to Trust as the Trust may request (at the Trust's cost); and

(c) Unless otherwise determined by the IPMG as part of its consideration of an overall patent strategy, or pursuant to its evaluation of a notice pursuant to Clause 10.2(a), if the Trust (or any licensee of the Trust) is exploiting the relevant Programme Intellectual Property following exercise of its rights pursuant to Clause 12, the Trust may take such action as it shall consider to be necessary or appropriate at its discretion and expense to bring or defend an action on behalf of the relevant member of the PTC Group. PTC shall (and PTC shall procure that the relevant member of the PTC Group shall) give the Trust all reasonable assistance as the Trust may request (at the Trust's cost) in relation to such action, including granting the Trust the right to bring such action in PTC's name.

10.3 In the event that any enforcement or defence action whether by a member of the PTC Group and/or the Trust results in the recovery of legal costs and/or an award of damages, such sums shall be distributed in accordance with the following the following order of priority: (a) first, to reimburse each Party for all litigation costs in connection with such proceeding paid by that Party and not otherwise recovered (on a pro rata basis based on each Party's respective litigation costs, to the extent the recovery was less than all such litigation costs); and (b) second, [**] percent ([**]%) to the enforcing Party and [**] percent ([**]%) to the non-enforcing Party.

10.4 Notwithstanding the above, except as may be agreed otherwise by the Parties following good-faith discussions, no Party shall enforce their rights under any Programme Intellectual Property for infringement or potential infringement by:

(a) any organisation operating on a Not for Profit Basis or any charitable organisation which is conducting non-commercially sponsored research; and/or

(b) any person carrying out non-commercially sponsored research on behalf of any organisation operating on a Not for Profit basis or any charitable organisation.

11. EXPLOITATION

11.1 To the extent that PTC finds it necessary or useful to acquire or license rights from Third Parties in order to use the Programme Intellectual Property for Development and Exploitation in the Field, PTC shall use commercially reasonable efforts to ensure that it has, or has the right to acquire, the ability to grant such rights to the Trust if the Trust becomes the Exploiting Party. For the avoidance of doubt, nothing in this Clause 11.1 shall be construed as a warranty or representation that any Products will be launched or that the use of Programme Intellectual Property for Development and Exploitation will not infringe any Third Party rights.

11.2 Each Party agrees that it shall promptly, (and in the case of PTC, will procure that the relevant member of the PTC Group shall promptly), inform and deliver written details to the other Parties of, any safety concerns or issues raised by any Competent Authority that relate to the Products.

11.3 Prior to any member of the PTC Group (whether itself or through any other member of the PTC Group, or by granting a license or in collaboration with any Third Party) (the "**Exploiting Party**"), commencing the Development and/or Exploitation of any Programme Intellectual Property and/or Products both inside and outside the Field, PTC or the relevant the member of the PTC Group shall obtain the prior written consent of the Trust to such Development and Exploitation by sending written notice to the Trust and the following information:

(a) reasonable details of the relevant Programme Intellectual Property, the Products and the activity proposed;

(b) details of whether the proposed Exploitation will be on a For-Profit and/or Not-For-Profit Basis; and

(c) if applicable, amounts of any milestones payments and royalties that would be payable to the Trust pursuant to Schedule 6 and any other applicable terms.

11.4 Where Exploitation is to be on a For-Profit Basis, the grant of the Trust's consent pursuant to Clause 11.6 shall be conditional on the payments to the Trust of amounts calculated pursuant to Schedule 6, and the Trust and PTC agreeing an appropriate share of any revenue payable to the Trust pursuant to Schedule 6.

11.5 In the event that the relevant member of the PTC Group Develops and/or Exploits the Programme Intellectual Property and/or Products in the Field on a Not for Profit Basis, no amounts shall be payable to the Trust in respect of such Development and/or Exploitation.

11.6 The Trust shall notify PTC in writing within [**] days of the receipt of the notice from PTC as to whether it consents (such consent not to be unreasonably withheld) to the Development and Exploitation of the Programme Intellectual Property and/or Products inside or outside the Field. Following receipt of such consent, PTC shall be free to Develop and Exploit the relevant Programme Intellectual Property in accordance with the consent given by the Trust without further consent or approval from the Trust. If, in respect of any Programme Intellectual Property, the Trust does not give its consent, the Parties shall meet to discuss the Trust's concerns and if they are unable to resolve those concerns the matter shall be referred to the dispute resolution procedure set out in Clause 19. All agreements entered into by PTC relating to the Programme Intellectual Property shall be consistent with the terms of this Agreement.

11.7 If PTC (either by itself, through any other member of the PTC Group, or through a Distributor) decides, at its own discretion not to Develop and Exploit any discrete part of the Programme Intellectual Property, PTC shall take reasonable steps to identify potential Licensees of the Programme Intellectual Property to Develop and Exploit Products. If neither PTC nor any other member of the PTC Group (either by itself or through a Distributor or

Licencee) takes reasonable steps to Develop and Exploit the Programme Intellectual Property, that Trust shall have the rights set out in Clause 12.

11.8 The Parties acknowledge that the Exploiting Party or any licensee of the Exploiting Party may be liable to pay royalties and make other payments to Third Parties (including members of the PTC Group) in respect of the Development and Exploitation of the Programme Intellectual Property and/or Products. The Exploiting Party agrees that it or the relevant licensee shall be solely responsible, at their own cost, for all such payments to Third Parties and any amounts payable to the other Parties under this Agreement shall not be reduced as a consequence except as explicitly provided in Schedule 6.

11.9 During the Programme Term, PTC shall keep the Trust reasonably informed on all matters relating to the Development and Exploitation of the Programme Intellectual Property and Products by or on behalf of PTC via the [**] RSG reports required pursuant to Clause 5.7. Following the Programme Term, PTC shall provide all matters relating to the Development and Exploitation of the Programme Intellectual Property and Products by or on behalf of PTC [**].

12. **TRUST STEP-IN RIGHTS**

12.1 Subject to Clause 12.4:

(a) if no member of the PTC Group or sub-licensees of the PTC Group is taking reasonable steps to Develop or Exploit any Programme Intellectual Property or Products for a particular indication for a consecutive period of [**] months or more following completion of the Programme, and, upon receipt of a written notice from the Trust served at the end of, or after such [**] month period requesting that the Programme Intellectual Property is Developed and/or Exploited, does not for an additional [**] months take any reasonable steps in this regard; or

(b) at any time after the first sale of a Product in a Major Market in a particular indication, no PTC Group member or any sub-licensees of any PTC Group member have taken reasonable steps to Develop and/or Exploit that Product in that particular indication in another Major Market for a consecutive period of [**] months, and, upon receipt of written notice from the Trust after such [**] month period requesting that the relevant Product is Exploited in such other region(s) and in such indication, does not for an additional [**] months take reasonable steps in this regard;

then, following the expiry of the time periods set out above, the Trust shall have the option in its sole discretion by giving written notice to PTC Therapeutics to become the Exploiting Party and to take responsibility for the Development and Exploitation of such Programme Intellectual Property and Products in the relevant indication(s) and region(s), which includes discretion to make any and all decisions (in consultation with the RSG) regarding the negotiation, acceptance and conclusion of terms for any agreement regarding the Development and Exploitation of such unexploited Programme Intellectual Property (including Development and Exploitation by way of licence, sale, materials transfer or other transfer of rights, as well as any transaction which

involves placing such unexploited Programme Intellectual Property into a separate corporate vehicle) in such region.

12.2 If the Trust exercises its right to exploit any Programme Intellectual Property under Clause 12.1:

(a) PTC will exclusively licence to the Trust or its nominee, the Programme Intellectual Property in such indications or regions as are specified in the notice served by the Trust exercising the option consistent with the applicable sub-clause in Clause 12.1. The terms of such exclusive licence to the relevant Programme Intellectual Property shall:

(i) be free of consideration in respect of sales of Product made on a Not-for-Profit Basis, and

(ii) include a share of any revenue or other consideration received by the Trust under any license of relevant Product Intellectual Property with respect to all other sales, such share to be based on the respective contributions made by PTC and the Trust in the Development and Exploitation of such Product;

(b) PTC will grant to the Trust or its nominee, a non-exclusive licence to relevant Background Intellectual Property solely as required and for the purposes of enabling the Trust to exercise the rights to the relevant Programme Intellectual Property as described in (a) above and solely in the regions specified in the notice served by the Trust exercising the option. Any such licence grant shall be non-exclusive and free of charge other than for reasonable costs that are incurred in respect of necessary third-party licences; and

(c) provide the Trust with access to any associated data, Documents (including, without limitation, Documents relating to pre-clincial data and clinical trials), pre-clinical data, Materials (only to the extent actually in existence and amenable to transfer in reasonable quantities without further regulatory approval(s), and not to include commercial inventory of Product for which PTC retains rights to Exploit), regulatory approvals, Marketing Approvals, or information as required for the Trust to exploit such rights.

12.3 If the Trust exercises its right to exploit any Programme Intellectual Property under Clause 12.1 above, PTC agrees that it shall pass (or will procure that relevant members of the PTC Group shall pass) to the Trust immediately any or all exploitation opportunities in the applicable region(s) that it becomes aware of from time to time in connection with the Programme Intellectual Property. PTC further undertakes that it shall not (and that it shall procure that no member of the PTC Group shall) engage in any activities (including in relation to the Background Intellectual Property) that could reasonably lead to the loss of an exploitation opportunity in the applicable region and with respect to the applicable indication(s) without the prior written consent of the Trust.

12.4 Notwithstanding anything to the contrary set forth in this Clause 12, in the event that PTC or a member of the PTC Group licenses a Third Party to

exploit the Programme Intellectual Property (whether alone or together with other Intellectual Property of any member of the PTC Group) in any indications and in any regions then the Trust shall have no rights under this Clause 12 with respect to such Programme Intellectual Property in such indications and in such regions, where:

(a) under a written agreement with a member of the PTC Group, such licensee is required to use diligent efforts to exploit the licensed Programme Intellectual Property in the relevant indication in the relevant region(s), and such written agreement provides for a reversion to the relevant member of the PTC Group of the Programme Intellectual Property in such indication and in such region(s) if the licensee materially breaches this diligence obligation; or

(b) the Trust has approved such licence in writing.

12.5 The Exploiting Party shall determine the regulatory plans and strategies and clinical trials ("**Key Product Strategy**") for any Products and shall be responsible for filing all regulatory filings with respect to the Products and will be responsible for obtaining and maintaining regulatory approvals in the name of the Exploiting Party. The Exploiting Party shall keep the Non-Exploiting Party informed regarding the Key Product Strategy for each Product and take into account the reasonable recommendations of the Non-Exploiting Party relating to such Key Product Strategy. Notwithstanding the above, the Parties acknowledge and agree that if the Non-Exploiting Party can reasonably demonstrate that any aspect of the Key Product Strategy proposed by the Exploiting Party will materially prejudice the Exploitation of any Product(s) which have been launched, the Exploiting Party will not proceed with such aspect of the Key Product Strategy.

13. REVENUE PAYMENTS

13.1 Unless otherwise agreed between the Parties in writing, all payments due to the Trust shall be made in to the following account:

Account Name:	The Wellcome Trust
Bank name:	[**]
Bank Address:	[**]
Sort Code:	[**]
Account No:	[**]
IBAN:	[**]
BIC:	[**]

13.2 Except as expressly provided herein or otherwise agreed between the Parties in writing, all payments due to PTC under this Clause 13 shall be made in to the following account:

Account Name:	PTC Therapeutics, Inc.
Account No.:	[**]
ABA No.:	031201467
Swift No.:	PNBPUS33
Bank:	Wachovia Bank NA
Branch address:	MAC N 2684-020, 120 Mountain View Blvd., Suite 200, Basking Ridge, NJ 07920, USA.

13.3 Within [**] days of the end of each Quarter, the paying Party shall deliver a statement to other Party setting out all sales of Product made by the paying Party, any member of the paying Party's Group or any Third Party in the relevant Quarter and the amount of revenue and any payment under Clauses 12 and 13 which is due to the receiving Party (**"Quarterly Statement"**). The receiving Party shall deliver to the paying Party an invoice for the amount due as set out in the Quarterly Statement in United States dollars. The revenue amount and any other amount invoiced shall be payable to the receiving Party within [**] days of receipt of the invoice.

13.4 With respect to amounts invoiced in United States dollars, all such amounts shall be expressed in United States dollars and shall be payable in United States dollars. With respect to amounts invoiced in a currency other than United States dollars, all such amounts shall be expressed, for information purposes only, in United States dollars as well as in the currency in which the amount was invoiced and shall be payable in the currency in which the amount is invoiced. The United States dollars equivalent shall be calculated using the paying Party's then current standard exchange rate methodology applied in its external reporting or the conversion of foreign currency sales into United States dollars, in each case as applied consistently throughout the paying Party's organisation.

13.5 If a Party does not receive payment of any sums due to it under this Clause 13 within the time specified, interest shall accrue on such sums at the rate equivalent to US LIBOR 3 months + [**], calculated on a daily basis.

13.6 All payments due under this Clause 13 are expressed to be exclusive of goods, sales, value added or any similar tax (**"Value Added Tax"**) howsoever arising, and the paying Party shall pay the receiving Party, in addition to those payments, all Value Added Tax for which the receiving Party is liable to account to any Competent Authority in relation to any supply made or deemed to be made for Value Added Tax purposes pursuant to this Agreement. The paying Party shall pay any payments due to the receiving Party under this Clause 13.6 at the same time as the relevant payment is due under this Agreement.

13.7 The obligation of the Trust to pay PTC the Award Amount in accordance with Clause 2 and the obligation of PTC and the members of the PTC Group to pay the Trust the revenue and any other payments in accordance with Clauses 12 and 13 shall be material obligations of this Agreement for the purposes of Clause 20.2 (a).

14. AUDIT OF REVENUE DUE

14.1 The Exploiting Party shall keep legible, true and accurate records and books of account for [**] years following the end of the calendar year to which they relate and procure that any affiliate of the Exploiting Party which is Exploiting the Programme Intellectual Property and any licensees of the Programme Intellectual Property shall keep legible, true and accurate records and books of account for [**] years following the end of the calendar year to which they relate, which contain all data necessary for the calculation of the revenue payable by it to any other Party (the "**Books**").

14.2 The Non-Exploiting Party shall have the right for a period of [**] years after receiving any report or statement with respect to royalties due and

payable to appoint an internationally-recognized independent accounting firm (the "**Auditor**") reasonably acceptable to the Exploiting Party to inspect the Books to verify such reports, statements, records or books of accounts, as applicable. Before beginning its audit, the auditor shall execute an undertaking acceptable to the Party being audited by which the auditor shall keep confidential all information reviewed during such audit. The auditor shall have the right to disclose to both the Party arranging the audit and the Party whose books have been audited, its conclusions regarding any payments owed to such Party.

14.3 The audited Party shall (and shall procure that its Affiliates and any licensees of the Programme Intellectual Property shall) make their records available for inspection by such auditor during regular business hours at such place or places where such records are customarily kept, upon receipt of reasonable advance notice from the Party arranging the audit, solely to verify the accuracy of sales reports, payments records or books of accounts and compliance in other respects with this Agreement. Such inspection right shall not be exercised more than [**] in any calendar year. The Party arranging the audit agrees to hold in strict confidence all information received and all information learned in the course of any audit or inspection, except to the extent necessary for such Party to reveal such information in order to enforce its rights under this Agreement or if disclosure is required by law, regulation or judicial order.

14.4 The Party arranging for the audit shall pay for such inspections, as well as its own legal expenses associated with enforcing its rights with respect to any payments hereunder, except that in the event there is any upward adjustment in aggregate amounts payable for any year shown by such inspection of more than [**] per cent ([**]%) of the amount paid, in which case the audited Party shall pay for such inspection.

15. PUBLICATIONS

15.1 So as not to jeopardise any Programme Patent filing or exploitation activity being undertaken, PTC shall (and shall procure that any member of the PTC Group or Licencee shall) provide the Trust with copies of any proposed publication or presentation which relates to a Programme Invention or Programme Intellectual Property in advance of the submission of such proposed publication or presentation to a journal, editor or publication. The Trust shall have at least [**] Business Days from and including the date of receipt from PTC of any proposed publication or presentation to object to the same because there is patentable subject matter relating to the Programme Invention that needs protection or because such publication would materially jeopardise any Exploitation activity. The Trust will not seek to withhold consent where such publication or presentation will not prejudice the protection or Exploitation of the Programme Intellectual Property and/or the Products.

15.2 In the event that the Trust objects to any such publication or presentation on the basis that it would disclose patentable information, PTC shall refrain (and shall procure that members of the PTC Group, any licensees, the Principal Investigator and the Staff also refrain), from making such publication or presentation for a period of [**] days from date of receipt of such objection in order for PTC to file the relevant patent application(s) with respect to the patentable subject matter contained in the proposed publication or

presentation. Following the expiry of such [**] day period or, if earlier, publication of any patent filed by PTC**,** PTC shall have the right to publish and reproduce any such publication freely with due acknowledgement of the source.

15.3 A copy of the final manuscript of all research publications that relate to the Programme must be made available from PubMed Central (or UK PubMed Central) as soon as possible and in any event no later than [**] months after publication.

16. ANNOUNCEMENTS

16.1 Save for the information described in Clause 16.2 or as required by law or any competent regulatory authority no announcement concerning this Agreement or its subject matter shall be made by the Parties without the prior written approval of both Parties. For clarity, once an item of information concerning this Agreement or its subject matter becomes public in compliance with the terms of this Agreement (for example, by an agreed press release or scientific publication), it may be used in public communications by either Party without the need for consent of the Parties.

16.2 The Trust may publish summary details of the Programme including the name of the Principal Investigator, the name of PTC, the title of the Programme, the Award Amount and the following description of the Programme:

"PTC Therapeutics, Inc. (PTC) is working to develop a novel drug that will target a protein called Bmi-1, a well-established oncogene that has been shown to be overexpressed in tumour cells and necessary for cancer stem cell survival. By inhibiting Bmi-1 expression, PTC anticipates making resistant cancer stem cells susceptible to treatment. Recent studies have also demonstrated that tumours have a sub-population of cells referred to as "cancer stem cells" that are involved in initiating tumour growth and progression. In addition, these stem-like cells are more resistant to chemical and radiation therapies than are other tumour cells. Although a large portion of the tumour can be debulked by chemo- or radio-therapies, the stem-like resistant cells are a pool of cells that are resistant to cancer therapies, ultimately causing tumour recurrence. Targeting Bmi-1 offers a strategy for therapeutic regimens intended to decrease these treatment failures, thereby improving patient outcome."

16.3 The Trust's contribution must be acknowledged in all scientific publications concerning the Programme, quoting the Award reference number.

17. CONFIDENTIALITY

17.1 Subject to Clauses 17.2 to 17.7 inclusive below, each Party undertakes that both during and for a period of [**] years after termination of this Agreement, it shall keep confidential and not disclose and shall take all reasonable security precautions to keep confidential and not disclose to any person other than to its officers, employees, consultants or professional advisors whose province it is to know, any Confidential Information of another party disclosed to or obtained by it in connection with this Agreement.

17.2 PTC shall only disclose the Confidential Information to those of its Staff who need to know it strictly for the purposes of the Programme and the

administration of the Award, provided that they are bound by confidentiality and non-use obligations in respect of such Confidential Information and are first made aware of PTC's confidentiality obligations towards the Trust.

17.3 If PTC considers it necessary for the purpose of the Programme to disclose the Confidential Information to employees, officers, students, visiting academics, contractors, sub-contractors, independent consultants or Third Parties who are not members of PTC's Staff employed on the Programme, then before any such disclosure takes place PTC shall procure that each of the persons concerned are bound by confidentiality and non-use obligations in respect of such Confidential Information and are first made aware of PTC's confidentiality obligations towards the Trust.

17.4 The Exploiting Party shall be entitled to disclose any Confidential Information of PTC or Confidential Information generated during the Programme if it is reasonably necessary or desirable to do so in order to protect, Develop or Exploit the Programme Intellectual Property and/or Products.

17.5 Without prejudice to Clause 17.1, and save in the case of publication in which case the provisions of Clause 15 shall apply, the Parties shall each use reasonable endeavours to keep details of any Programme Inventions confidential pending filing of a patent application claiming such Programme Invention.

17.6 Clause 17.1 above shall not apply to:

(a) information which is or was already known to the receiving party at the time of disclosure under this Agreement, as shown by the receiving party's written records, without any obligation to keep it confidential;

(b) information which is independently developed by employees of the receiving party who have not had access to the confidential information of the disclosing party;

(c) information which at the time of being disclosed or obtained by the receiving party under this Agreement or at any time thereafter, is published or otherwise generally available to the public other than due to default by the receiving party of its obligations hereunder;

(d) the disclosure of information by the Trust for the purposes of publishing summary details of awards made by the Trust

(e) the disclosure of information for the purpose of registering a clinical trial on a national or international clinical trial register or on the Trust's clinical trial register or for the purpose of patient recruitment with respect to a clinical trial;

(f) the disclosure to a Party's professional advisers or to the Trust's Site Visit Group of information reasonably required to be disclosed for purposes relating to this Agreement.

17.7 Each Party shall ensure that all Staff, personnel and Third Parties to whom confidential information of the other party is disclosed are informed of the provisions of Clauses 15 (Publications), 16 (Announcements) and this Clause 17 (Confidentiality).

18. WARRANTIES AND INDEMNITIES

18.1 The Trust warrants that:

(a) it has the requisite authority to enter into this Agreement; and

(b) it has full power and authority to assume all of its obligations under this Agreement.

18.2 PTC represents and warrants to the Trust on the Commencement Date and immediately prior to the payment by the Trust to PTC of any Tranche (or installment thereof) that (subject to any matters fairly and accurately disclosed in the Disclosure Letter):

(a) it has the requisite authority to enter into this Agreement;

(b) it has full power and authority to assume all of its obligations under this Agreement;

(c) the Agreement has been duly authorised, executed, and delivered by PTC and is a valid, binding, and legally enforceable obligation of PTC;

(d) no consent, approval, authorisation, or order of any court or governmental agency or body is required for the consummation of the transactions contemplated by this Agreement;

(e) the execution, delivery, and performance of this Agreement will not result in a breach or violation of, or constitute a default under, any statute, regulation, or other law or agreement or instrument to which it is a party or by which it is bound, or any order, rule, or regulation of any court or governmental agency or body having jurisdiction over it or any of its properties;

(f) to the best of its knowledge and belief:

(i) PTC is the legal and beneficial owner of, or has appropriate license to, all right, title and interest in and to the Background Intellectual Property necessary for performance of the Programme, and will be the legal and beneficial owner of, or procure appropriate license or option rights to, all right, title and interest in and to the Programme Inventions and Programme Intellectual Property;

(ii) no member of the PTC Group has granted any Third Party any right in respect of the Programme Inventions or Programme Intellectual Property (other than in accordance with the terms of this Agreement), and has not charged or encumbered and will not charge or encumber any of the same except as may be explicitly authorised pursuant to this Agreement;

(iii) so far as PTC is aware, no Third Party has made unauthorised use of any Background Intellectual Property, nor threatened to do so;

(iv) so far as PTC is aware, none of the activities of any member of the PTC Group undertaken by prior to the date on which the warranties are given or which will be undertaken pursuant to the Programme relating to the Background Intellectual Property infringe, or have been alleged to infringe, the Intellectual Property of any Third Party;

(v) the Background Intellectual Property and Programme Intellectual Property are not subject to any claim, opposition, attack, assertion or other arrangements of whatever nature which may impugn upon the use, validity, enforceability or ownership of any such Intellectual Property, and there are no grounds or other circumstances which may give rise to the same;

(vi) From the Commencement Date forward, no member of the PTC Group has itself nor through any of its Staff disclosed to any Third Party (other than consistent with Clause 17) any Confidential Information and/or Know-How relating to the Programme;

(vii) Other than as required by law (including contracts with academic collaborators and government entites or in connection with the use of government funds) no person has, or will have, the right to call for the assignment or grant of the licence to it of any of the Background Intellectual Property and the Programme Intellectual Property under any option, grant, funding award or other agreement, nor is there any conditional or unconditional agreement or circumstance whereby such a right may arise;

(viii) no person has any right or claim to any payment or other compensation in respect of the use or exploitation of the Background Intellectual Property or the Programme Intellectual Property; and

(ix) there are no outstanding or potential claims against any member of the PTC Group under any contract or for employee compensation under applicable legislation in relation to the Background Intellectual Property nor is PTC aware of any reason why any such claims may be made in relation to the Programme Intellectual Property.

18.3 Except as expressly provided in this Agreement, neither Party gives any warranties or makes any representations with respect to any of the Programme Intellectual Property and/or Background Intellectual Property or any Products derived from them, or their fitness for any purpose, or that any material produced or supplied by any Party and any processes or techniques used, proposed or recommended by any Party will not infringe any patent or other Intellectual Property of any person in any country.

18.4 Subject to Clause 18.6 below, the Trust's maximum liability in aggregate to PTC arising out of this Agreement shall not exceed the Award Amount.

18.5 Except in circumstances of fraud or wilful misconduct by a Party or its Affiliates, no Party nor any of its Affiliates shall be liable to another Party or any Affiliate of another Party for special, indirect, incidental or consequential damages, whether in contract, warranty, negligence, tort, strict liability or otherwise, arising out of any breach of or failure to perform any of the provisions of this Agreement.

18.6 Nothing in this Agreement shall limit the liability of any Party in respect of:

(a) personal injury or death arising out of that Party's negligence or wilful misconduct, or

(b) fraud or wilful misconduct or fraudulent misrepresentation.

18.7 PTC shall be responsible for and indemnify and keep fully indemnified the Trust and its Affiliates, officers, servants, agents, sub-licensees and sub-sub-licensees (collectively the "**Trust Indemnified Parties**" and each a "**Trust Indemnified Party**") against any and all liability, loss, damage, cost or expense ("**Losses**") incurred or suffered by such Trust Indemnified Party as a result of any claim by a Third Party arising directly out of the Programme and/or the Development, use, promotion, marketing, sale, Exploitation or distribution of the Programme Intellectual Property and/or Products by, or on behalf of, PTC, except to the extent such Losses result from the negligence or intentional misconduct of the Trust Indemnified Party.

19. **DISPUTE RESOLUTION**

19.1 Any question, difference or dispute which may arise concerning the construction meaning or effect of this Agreement or concerning the rights and liabilities of the Parties hereunder or any other matter arising out of or in connection with this Agreement shall first be submitted to the Director of the Technology Transfer Division of the Trust and the General Counsel of PTC (or their designees) (the "**Senior Officers**"), who may call on others to advise them as they see fit.

19.2 If the Senior Officers are unable to resolve the dispute pursuant to Clause 19.1 within [**] Business Days of the date on which the matter is referred to them, such dispute may be referred by either Party for resolution by an independent chartered accountant (an "**Expert**") to be appointed (in default of nomination by agreement between the Trust and PTC) by the President for the time being (or next available senior officer) of the Institute of Chartered Accountants in England and Wales. The following provisions shall govern the appointment of the Expert:

(a) The Expert shall prepare a written decision and give notice (including a copy) of the decision to the Parties within a maximum of [**] Business Days of the matter being referred to him.

(b) If the Expert dies or becomes unwilling or incapable of acting, or does not deliver the decision within the time required by Clause 19.2 then:

(i) either Party may apply to the president of the Institute of Chartered Accountants in England and Wales to discharge the Expert and to appoint a replacement Expert with the required expertise; and

(ii) this Clause 19.2 shall apply in relation to the new Expert as if he were the first Expert appointed;

(c) The Parties shall be entitled to make submissions to the Expert including oral submissions and shall provide each Party with a copy of any such submissions and additionally shall provide (or procure that others provide) the Expert with such assistance and documents as the Expert reasonably requires for the purpose of reaching a decision.

(d) To the extent not provided for by this Clause 19, the Expert may, in his reasonable discretion, determine such other procedures to assist with the conduct of the determination as he considers just or appropriate.

(e) Each Party shall, with reasonable promptness, supply each other with all information and give each other access to all documentation and personnel as each other reasonably requires to make a submission under this Clause 19.

(f) The Expert shall act as an expert and not as an arbitrator. The Expert shall determine any dispute, which may include any issue involving the interpretation of any provision of this Agreement, his jurisdiction to determine the matters and issues referred to him or his terms of reference. The Expert's written decision on the matters referred to him, if accepted by the Parties, shall be final and binding in the absence of manifest error or fraud; provided, however that either Party in its sole discretion may decline to accept the Expert's written decision and instead refer the dispute to arbitration pursuant to Clause 19.3.

(g) Each Party shall bear its own costs in relation to the Expert. The Expert's fees and any costs properly incurred by him in arriving at his determination (including any fees and costs of any advisers appointed by the Expert) shall be borne by the Parties equally or in such other proportions as the Expert directs.

19.3 If the procedure under Clauses 19.1 and 19.2 should fail to resolve the question, difference or dispute (including any question regarding the existence, validity or termination of this Agreement) the Parties agree to proceed to binding arbitration. Unless otherwise agreed by the Parties, the arbitration will be take place in London, England, according to the rules of the London Court of International Arbitration ("**LCIA Rules**"), which LCIA Rules are deemed to be incorporated by reference into this clause, except to the extent such rules are inconsistent with this Clause 19.3. The Parties shall bear their own costs of counsel and other professional advisers in such arbitration, regardless of outcome, and the Parties shall share equally in the cost of the arbitration. The arbitration will be conducted by one (1) arbitrator who shall be reasonably acceptable to the Parties and who shall be appointed in accordance with LCIA Rules. If the Parties are unable to select an arbitrator, then the arbitrator shall be appointed by the LCIA. Any arbitrator chosen hereunder shall have educational training and industry experience sufficient to demonstrate a reasonable level of scientific, financial, medical and industry knowledge relevant to the particular dispute. If the question, difference or dispute relates to existence, validity or termination of this Agreement, then the arbitrator shall resolve the question, difference or dispute, and the arbitration shall be conducted according to LCIA Rules. If

the question, difference or dispute relates to any other matter under this Agreement, then the arbitration shall be conducted according to the following rules:

(a) Within [**] Business Days after the selection of the arbitrator, each Party shall submit to the arbitrator and the other Party a proposed resolution of the dispute that is the subject of the arbitration, together with any relevant evidence in support thereof (the "Proposals"). Within [**] Business Days after the delivery of the last Proposal to the arbitrator, each Party may submit a written rebuttal of the other Party's Proposal and may also amend and re-submit its original Proposal. The Parties and the arbitrator shall meet within [**] Business Days after the Parties have submitted their Proposals, at which time each Party shall have [**] to argue in support of its Proposal. The Parties shall not have the right to call any witnesses in support of their arguments, nor compel any production of documents or take any discovery from the other Party in preparation for the meeting. Within [**] Business Days after such meeting, the arbitrator shall select one of the Proposals so submitted by one of the Parties as the resolution of the dispute, but may not alter the terms of either Proposal and may not resolve the dispute in a manner other than by selection of one of the submitted Proposals. If a Party fails to submit a Proposal within the initial [**] Business Day time frame set forth in the first sentence of this Clause 19.3(a), the arbitrator shall select the Proposal of the other Party as the resolution of the dispute. Any time period set forth in this Clause 19.3(a) may be extended by mutual agreement of the Parties.

19.4 The results of an arbitration pursuant to Clause 19.3 shall be binding and enforceable against the Parties in any court of competent jurisdiction, and the Parties hereby consent to the jurisdiction of the English courts for such purpose.

19.5 Notwithstanding the foregoing provisions of Clause 19.3, either Party will have the right to seek interim or provisional relief in any court of competent jurisdiction as may be available to such Party under the laws and rules applicable in such jurisdiction, including a temporary restraining order, preliminary injunction or other interim equitable relief, concerning a dispute either prior to or during arbitration under Clause 19.3, if necessary to protect the interests of such Party or to preserve the status quo pending final arbitration.

20. DURATION AND TERMINATION

20.1 This Agreement shall commence on the Commencement Date and shall continue for whichever is the longer of:

(a) the term of the funding under this Agreement and, if applicable, any further funding granted by the Trust in connection with or as a result of the Programme;

(b) the period that the Programme takes to complete;

(c) the last to expire of the Programme Patents;

(d) the expiry of any agreement entered into for the exploitation of the Programme Intellectual Property or the Background Intellectual Property; or

(e) the expiry of any payment obligation relating to the exploitation of the Programme Intellectual Property or the Background Intellectual Property.

20.2 Each Party ("**Terminating Party**") shall have the right to terminate this Agreement forthwith at any time upon giving written notice of termination to the other Party (the "**Defaulting Party**"), upon the occurrence of any of the following events:

(a) the Defaulting Party commits a breach of a material obligation set out in this Agreement which is not capable of remedy;

(b) the Defaulting Party commits a breach of a material obligation set out in this Agreement which is capable of remedy but has not been remedied within [**] Business Days of the receipt by it of a notice from the Terminating Party identifying the breach and requiring its remedy;

(c) the Defaulting Party is unable or admits inability to pay its debts as they fall due, suspends making payments on any of its debts or, by reason of actual or anticipated financial difficulties commences negotiations with one or more of its creditors with a view to rescheduling any of its indebtedness;

(d) a proposal is made or a nominee or supervisor is appointed for a composition in satisfaction of the debts of the Defaulting Party or a scheme or voluntary arrangement of its affairs within the meaning of the relevant bankruptcy or insolvency laws, or the Defaulting Party enters into any composition or voluntary arrangement for the benefit of its creditors, or proceedings are commenced in relation to the Defaulting Party under any law, regulation or procedure relating to the re-construction, deferment or re-adjustment of all or substantially all of the Defaulting Party's debts;

(e) the Defaulting Party takes any action, or any legal proceedings are started whether by a Third Party or not, for the purpose of the winding up or dissolution of the Defaulting Party, other than for a solvent reconstruction or amalgamation;

(f) the appointment of a liquidator, trustee, receiver, administrative receiver, receiver and manager, interim receiver custodian, sequestrator, administrator or similar officer, in respect of all or a substantial part of the assets of the Defaulting Party;

(g) an effective resolution being passed for the winding-up or entering into administration (whether out of court or otherwise) of the Defaulting Party;

(h) a distress, execution or other legal process being levied against all or substantially all of the assets of the Defaulting Party, and not being

discharged or paid out in full within [**] Business Days of the commencement of each process; or

(i) the occurrence in respect of the Defaulting Party of any event in any jurisdiction to which it is subject having an effect similar to that of any of the events referred to in Clauses 20.2 (c) to 20.2 (h) above.

20.3 In addition, the Trust shall be entitled to terminate this Agreement by notice in writing to PTC, such termination to take effect as specified in the notice, if:

(a) During the Programme Term, PTC fails to comply with any of the Conditions and where such non-compliance is capable of remedy, PTC has not remedied it within [**] Business Days of the receipt by it of a notice from the Trust identifying the non-compliance and requiring its remedy; or

(b) During the Programme Term, the Site Visit Group recommends termination of the Programme in accordance with Clause 6 and PTC fails to correct any identified failings within the applicable time period under Clause 6.3;

(c) PTC ceases or threatens to cease to carry on all or a substantial part of its business or operations necessary for the completion of its obligations under this Agreement;

(d) PTC takes any action, or omits to take any action, the consequences of which, in the reasonable opinion of the Trust, would be incompatible with or have an adverse effect:

(i) on the Trust's charitable objectives or reputation; or

(ii) on the ability of PTC to comply with its respective obligations under this Agreement; and/or

(e) PTC enters into transactions involving any of the Programme Intellectual Property and/or Background Intellectual Property without the prior written consent of the Trust, including, without limitation, assigning or otherwise transferring any Programme Intellectual Property or Background Intellectual Property or any interest in such Intellectual Property to an Affiliate or Third Party and/or creating any new security or increasing any existing security over any of the Programme Intellectual Property and/or Background Intellectual Property (other than netting or set-off arrangements entered into in the ordinary course of PTC's banking or financing arrangements for the purpose of netting debit and credit balances; or any lien arising by operation of law and in the ordinary course of business).

20.4 If the during the Programme Term the Principal Investigator ceases to be involved with the Programme, ceases to be employed by or provide services to PTC, ceases to carry out research at premises controlled by PTC, or is prevented through illness or injury from promptly fulfilling his obligations under this Agreement, the Trust shall consult with PTC to ascertain whether the Programme or its progress will be jeopardised by such event. If in the reasonable opinion of the Trust:

(a) such event will jeopardise the Programme or its progress, and the Parties after good-faith negotiations are unable to agree on a replacement Principal Investigator, the Trust may terminate this Agreement by written notice (provided, that such determination and termination by the Trust shall be final and binding and shall not be subject to the dispute resolution or arbitration procedures set forth in Clause 19); or

(b) the Programme has reached a stage such that the services of the Principal Investigator are not key to the completion of the Programme, the Trust and PTC shall negotiate in good faith any amendments necessary to this Agreement so as to enable the satisfactory completion of the Programme within a reasonable time.

20.5 In the event that PTC undergoes a Change of Control, PTC shall give the Trust prompt notice of such Change of Control.

(a) During the Programme Term, if in the Trust's reasonable opinion, the Change of Control would have an adverse effect on, or be incompatible with the Trust's charitable objectives or PTC's ability to fulfil its obligations under the Agreement, the Trust may in its absolute discretion, terminate the Agreement by serving written notice of termination on PTC.

(b) Following the Programme Term, the surviving entity following such Change of Control shall, within [**] days of such Change of Control, confirm in writing to Trust its intentions to continue to meet its obligations under this Agreement, and meet with Trust to present its plans for Exploiting the Programme Intellectual Property and commercializing Products.

(i) If no Product resulting from the Programme is being tested in Phase 1 or later trials or has received Marketing Approval, and if following the meeting contemplated by Clause 20.5(b), in the Trust's reasonable opinion, the Change of Control would have an adverse effect on, or be incompatible with the Trust's charitable objectives or the ability of PTC's successor in interest to fulfil its obligations under the Agreement, the Trust may in its absolute discretion, terminate the Agreement by serving written notice of termination on such successor in interest. For clarity, If any Product resulting from the Programme is being tested in Phase 1 or later trials, and following the meeting contemplated by Clause 20.5(b), the Trust shall not have any termination rights under this Clause 20.5(b)(i), but the Trust and PTC's successor in interest shall negotiate in good faith for a resolution of the issues raised by the Trust, and any failure to reach an agreement on such resolution within a reasonable time period may be referred in the Trust's sole discretion to the dispute resolution and arbitration procedures specified in Clause 19.

(ii) If PTC's successor in interest fails to provide the written notice or refuses to hold the meeting contemplated in Clause 20.5(b), then the Trust may in its absolute discretion,

terminate the Agreement by serving written notice of termination on PTC's successor in interest.

21. **EFFECT OF TERMINATION**

21.1 Termination of this Agreement howsoever arising shall be without prejudice to the rights and duties of any Party accrued prior to termination. Except as may be otherwise provided in this Clause 21, the Clauses in this Agreement which expressly have effect after or notwithstanding termination (including without limitation Clauses 1, 2.10, 2.11, 2.13, 4, 8, 9 10, 11, 12, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, 28, 29 and 30) shall continue to be enforceable notwithstanding termination.

21.2 Upon termination prior to the end of the Programme, PTC shall return all funding received from the Trust under this Agreement which is unspent at the date of termination (after deduction of costs and non-cancellable commitments incurred prior to the date of termination).

21.3 On termination of this Agreement by the Trust in accordance with Clauses 20.2 or 20.5(b)(ii); PTC shall, so far as it is able to do so without violating legal requirements or breaching contractual obligations that existed prior to the event giving grounds for termination:

(a) for no consideration assign all of its rights in the Programme Intellectual Property to the Trust or a Third Party nominated by the Trust;

(b) for no consideration procure that any licences of Programme Intellectual Property granted to PTC shall be assigned to the Trust or a Third Party nominated by the Trust or sub-licensed to the Trust or a Third Party nominated by the Trust on a world-wide, perpetual basis. Such sub-licenses shall be: (a) non-exclusive to complete the Programme and exclusive in relation to Development and Exploitation; and (b) free of charge and royalty free.

(c) upon request from the Trust and at no charge to the Trust, provide such assistance to the Trust as the Trust may reasonably require to assist in the assignment or sub-licensing of the rights in the Programme Intellectual Property or any licences pursuant to this Clause 21.3 and/or in the closure of the Programme;

(d) upon request from the Trust:

(i) grant to the Trust as requested by the Trust a world-wide, royalty free, perpetual, non-exclusive licence to use any and all of the PTC Background Intellectual Property owned or sub-licensable by PTC or any member of the PTC Group and required for further research in accordance with the Programme and/or Development and Exploitation of Programme Intellectual Property; and

(ii) discuss in good faith a worldwide, non-exclusive licence to use any and all of the PTC Background Intellectual Property owned or sub-licensable by PTC or any member of the PTC Group for additional research, Development and Exploitation;

(e) provide to the Trust with all laboratory notebooks and other records relating to the Programme Intellectual Property and the Programme Books and Records;

(f) as requested by the Trust, carry out a hand over of the Programme to the Trust or wind down the Programme for a reasonable period of time, such period not to exceed [**] months following termination; and

(g) return all equipment acquired by PTC using the Trust Award.

21.4 On termination of this Agreement by the Trust in accordance with Clauses 20.3, 20.4, 20.5(a), or 20.5(b)(i), PTC shall meet with Trust to in good faith to address mitigation of any harm to Trust resulting from PTC's actions giving rise to the termination right, and any failure to reach an agreement on such resolution within [**] days may be referred in the Trust's sole discretion to the dispute resolution and arbitration procedures specified in Clause 19. For clarity, this Clause 21.4 shall not be interpreted to limit Trust's ability to seek additional remedies available under this Agreement or otherwise at law with respect to the events giving rise to the applicable termination right.

21.5 On termination of this Agreement by PTC in accordance with Clause 20.2, notwithstanding any other provision of this Agreement, all of the Trust's rights under Clauses 2, 3, 4, 5, 6, 7, 8, 9, 10, 11, 12, 13, 14 and 15, as well as any rights to receive payments pursuant to Schedule 6, shall be extinguished and of no further force and effect. For clarity, this Clause 21.5 shall not be interpreted to limit PTC's ability to seek additional remedies available under this Agreement or otherwise at law with respect to the events giving rise to the applicable termination right.

22. **WAIVER**

22.1 Neither Party shall be deemed to have waived any of its rights or remedies under this Agreement unless the waiver is expressly made in writing and signed by a duly authorised representative of that Party. In particular, no delay or failure of a Party in exercising or enforcing any of its rights or remedies under this Agreement shall operate as a waiver of those rights or remedies nor shall any single or partial exercise or enforcement of any right or remedy by a Party preclude or impair any other exercise or enforcement of that right or remedy by that Party.

23. **ENTIRE AGREEMENT/VARIATIONS**

23.1 This Agreement, together with the Application and any agreement entered into pursuant to the Agreement constitutes the entire agreement and understanding between the Parties relating to the subject matter hereof and together they supersede and replace all prior drafts, previous understandings, arrangements, representations or agreements, whether in writing or oral, between the Parties relating to the subject matter of this Agreement.

23.2 No variation, amendments, modification or supplement to this Agreement shall be valid unless and until it is made in writing and signed by a duly authorised representative of each of the Parties.

24. **ASSIGNMENT**

24.1 Except for a Change of Control in compliance with Clause 20.5, PTC shall not without the prior written consent of the Trust assign, transfer, convey or declare a trust over this Agreement or make any other disposition (whether in whole or in part) of any of its rights and obligations hereunder to any Third Party.

25. **SEVERANCE OF TERMS**

25.1 If the whole or any part of this Agreement is or becomes or is declared illegal, invalid or unenforceable in any jurisdiction for any reason (including both by reason of the provisions of any legislation and also by reason of any court or competent authority which either has jurisdiction over this Agreement or has jurisdiction over any of the Parties):

(a) In the case of the illegality, invalidity or un-enforceability of the whole of this Agreement it shall terminate only in relation to the jurisdiction in question; or

(b) In the case of the illegality, invalidity or un-enforceability of part of this Agreement that part shall be severed from this Agreement in the jurisdiction in question and that illegality, invalidity or un-enforceability shall not in any way whatsoever prejudice or affect the remaining parts of this Agreement, which shall continue in full force and effect.

25.2 If in the reasonable opinion of any Party any severance under this Clause 25 materially affects the commercial basis of this Agreement, the Parties shall discuss, in good faith, ways to eliminate the material effect.

26. **COSTS**

26.1 Each Party shall bear its own legal costs, legal fees and other expenses incurred in the preparation and execution of this Agreement.

27. **FURTHER ASSURANCES**

27.1 Each Party shall perform such acts and execute such documents as may be reasonably required for securing to or vesting in another Party the rights agreed to be granted to it under or pursuant to this Agreement.

28. **NOTICES**

28.1 Any notice to be given pursuant to this Agreement shall be in writing in the English language and shall be delivered by international courier, by registered, recorded delivery or certified mail (postage prepaid) or by facsimile confirmed by registered, recorded delivery or certified mail (postage prepaid) to the address or facsimile number of the recipient Party set out below or such other address or facsimile number as a Party may from time to time designate by written notice to the other Parties. Any notice by facsimile

shall be confirmed by the sender sending a confirmatory copy of the notice by registered, recorded delivery or certified mail (postage prepaid).

Address of PTC

PTC Therapeutics, Inc.

100 Corporate Court
South Plainfield, NJ 07080
United States

Fax No:	+1 (908) 222-1128
for the attention of:	Legal Department
With an email copy to	legal@ptcbio.com

Address of the Trust

Technology Transfer Division
The Wellcome Trust Limited
215 Euston Road
London NW1 2BE

Fax No:	+44 (0) 20 7611 8857
for the attention of:	[**]
with a copy to:	[**]

28.2 Any notice given pursuant to this Clause 28 shall be deemed to have been received:

(a) in the case of delivery by international courier or sending by certified mail, on the day of receipt, provided receipt occurs on a Business Day of the recipient Party or otherwise on the next following Business Day of the recipient; or

(b) in the case of facsimile, on acknowledgement by the recipient facsimile receiving equipment on a Business Day if the acknowledgement occurs before 5:00 pm local time of the recipient Party and in any other case on the following Business Day.

28.3 Any notice that is required in this Agreement may be validly given if transmitted by fax or sent by post in accordance with this Clause 28. Email alone is not a valid method of giving notice under this Agreement.

29. **GENERAL**

29.1 Nothing in this Agreement shall be taken to constitute a partnership between the Parties. Except as specifically provided in this Agreement, none of the Parties shall by reason of this Agreement be empowered to act as agent for any other party nor to pledge the credit of any other party nor shall any Party be held liable for or incur liability in respect of the acts or defaults of any other Party to this Agreement.

29.2 This Agreement may be executed in any number of counterparts and by the Parties on separate counterparts, but shall not be effective until each Party has executed at least one counterpart. Each counterpart shall constitute an original of this Agreement, but all the counterparts shall together constitute one and the same instrument.

29.3 A person who is not a Party has no right under the Contracts (Rights of Third Parties) Act 1999 to enforce or to enjoy the benefit of any term of this Agreement.

30. **GOVERNING LAW**

30.1 This Agreement (and any dispute, controversy, proceedings or claim of whatever nature arising out of this Agreement or its formation) shall be governed by and construed in accordance with the laws of England. The Parties irrevocably submit to the exclusive jurisdiction of the Courts of England provided that nothing in this clause shall prevent any Party from seeking injunctive relief in any court of competent jurisdiction in respect of a breach or threatened breach of Clause 17 (Confidentiality).

IN WITNESS whereof the Parties or their duly authorised representatives have executed this Agreement on the date hereinbefore written.

SCHEDULE 1

THE PROGRAMME

[Insert copy of Application]

APPLICATION FOR A SEEDING DRUG DISCOVERY STRATEGIC AWARD

PLEASE READ THE WELLCOME TRUST'S GRANT CONDITIONS (www.wellcome.ac.uk/fundingpolicy)
AND INFORMATION FOR APPLICANTS (http://www.wellcome.ac.uk/assets/wtx027225.doc)
IN CONJUNCTION WITH THIS APPLICATION FORM.

WELLCOME TRUST DATA PROTECTION STATEMENT

Information that you supply to the Wellcome Trust in connection with this Application (which includes all information sent to the Wellcome Trust that relates to your application, or, in the event of an award, relates to that award) will be used to process your Application and for the purposes of audit and/or evaluation. It may also be disclosed to external peer reviewers, some of whom may be based outside the EEA. Your personal data will be stored by or on behalf of the Wellcome Trust, and/or organisations connected with it, in accordance with the Data Protection Act 1998. Where we fund in partnership with other organisations your personal data may also be disclosed to and processed by the partner(s) involved. The Wellcome Trust may publish basic details of successful awards (e.g. on its website or in its Annual Report) and/or anonymise your personal data for research and statistical purposes. The Wellcome Trust may also release details of successful awards (including your name and employing Institution, the project title, and the scientific abstracts and lay summaries of the research) into the public domain (e.g. via the internet or via publicly accessible databases). The Wellcome Trust may contact you about other award schemes and initiatives that may be of interest to you, or for your views on its funding schemes and application processes. Please contact the Wellcome Trust if you have any questions about the protection of your personal data.

UNDERTAKINGS

1. I confirm that I (and all those providing personal information in the application) have read and understood the Wellcome Trust Data Protection statement above.

2. To the best of my knowledge, the information provided in this application is accurate and complete.

3. I have read the conditions under which grants are awarded and, if a grant is made, I agree to abide by them.

4. The necessary facilities will be made available to conduct this research, and will continue to be available for the duration of the Wellcome Trust's award.

5. During the period of any funding, I will promptly inform the Wellcome Trust of any material changes to any details provided in this application.

Signature of Principal Applicant	[ILLEGIBLE]	Date:	2-23-10
Signature of Coapplicant (1)	[ILLEGIBLE]	Date:	2/23/10
Signature of Coapplicant (2)	[ILLEGIBLE]	Date:	2-23-10
Signature of Drug Discovery Advisor	[ILLEGIBLE]	Date:	2-23-10
Signature of Head of Technology Transfer Office/Group	[ILLEGIBLE]	Date:	2-23-10

Front page

For and on behalf of the Institution:

Signature of Secretary of Institution/Finance Officer/Company Official: /s/ Mark Boulding **Date:** 23 Feb-2010

Position: SVP and General Counsel **Company/ Institution:** PTC Therapeutics, Inc

Q1 Applicants	**Principal Applicant**	**Coapplicant(1)**	**Coapplicant (2)**	**Coapplicant (3)**	**Technology Transfer Officer**
Surname	Davis	Moon	Weetall		
Forenames	Thomas W.	Young-Choon	Maria L		
Title (Dr etc.)	Director of Biology, (Ph.D.)	Director Chemistry, (Ph.D.)	Director Pharmacology, (Ph.D.)		

Q2 **Title of project:** (no more than 220 characters)

Discovery and development of a novel glioblastoma treatment that selectively reduces Bmi-1 expression in tumour stem cells

Q3 **Company name and address or department name and address at administering institution:**

PTC Therapeutics, Inc.
100 Corporate Ct
South Plainfield, NJ 07080
USA

Q4	**Type of Translation Award requested:**	SEEDING DRUG DISCOVERY
Q5	**Period for which support is sought:** (state in months)	30
Q6	**Proposed start date:** (dd/mm/yy)	01/07/2010

Principal Applicant			
Name	Thomas W. Davis, Ph.D.	Telephone numbers:	
Contact address	PTC Therapeutics, Inc.	Day	[**]
	100 Corporate Court	Mobile	[**]
	South Plainfield, NJ 07079	Fax.	[**]
		e-mail	[**]
Coapplicant (1)			
Name	Young-Choon Moon, Ph.D.	Telephone numbers:	
Contact address	PTC Therapeutics, Inc.	Day	[**]
	100 Corporate Court	Mobile	
	South Plainfield, NJ 07079		
		Fax.	[**]
		e-mail	[**]
Coapplicant (2)			
Name	Marla L Weetall, Ph.D.	Telephone numbers:	
Contact address	PTC Therapeutics, Inc.	Day	[**]
	100 Corporate Court	Mobile	
	South Plainfield, NJ 07079		
		Fax.	[**]
		e-mail	[**]
Technology Transfer Officer			
Name	Don Mankoff	Telephone numbers:	
Contact address	PTC Therapeutics, Inc.	Day	[**]
	100 Corporate Court	Mobile	
	South Plainfield, NJ 07079		
		Fax.	[**]
		e-mail	[**]
Drug Discovery Advisor			
Name	Neil Almstead, Ph.D.	Telephone numbers:	
Contact address	PTC Therapeutics, Inc.	Day	[**]
	100 Corporate Court	Mobile	
	South Plainfield, NJ 07079	Fax.	[**]
		e-mail	[**]

Contact details

If a company please provide the following:

Company number:	908 222 7000
Date and place of incorporation:	31/03/1998 — Delaware
Shared capital:	
Authorised:	Preferred Stock-158,788,832 & Common Stock- 21,550,000
Issued:	Preferred Stock-153,356,396 & Common Stock- 129,306
Registered holders (name, number and type):	Investors: CSFB Private Equity, HBM Bioventures, Vulcan Ventures, Delphi Ventures, Celgene, Bay City Capital, Novo A/S, HealthCap, Pfizer, The Column Group, Novartis BioVentures, Genavent Partners LP, Gilead, Amgen Ventures, Manufacturers Life, Hansa Special Opportunities, Birchmere Ventures, Pictet Funds — Biotech and POSCO BioVentures
Registered office:	N/A
Directors:	CEO — Stuart W. Peltz, Ph.D. CFO — William Baird General Counsel/Secretary — Mark Boulding
Secretary:	Mark Boulding
Accounting reference date:	31/12/2009

Previous source of funding and amount:	[**]
Cash in bank and other investments:	[**]
Average monthly expenditure:	[**]
Board of Directors:	Michael Schmertzler, Axel Bolte, Soren Carlsen, Ph.D., Carl Goldfischer, M.D., Allan Jacobson, Ph.D., Michael Kranda, Deepa Pakianathan, Ph.D., Stuart W. Peltz, Ph.D., David P. Southwell

Details of Project

Scientific advisory Board:	Allan Jacobson, Ph.D., Eric N. Jacobsen, Ph.D., Paul A. Marks, M.D., Robert Schneider, Ph.D., Marvin Wickens, Ph.D., Joseph Puglisi, Ph.D.
Number of Employees:	187

Please enclose a copy of the current Business Plan Executive Summary with application.

Q7 TIME SPENT BY APPLICANTS ON RESEARCH

(a) How many hours per week do the Principal Applicant and Coapplicant(s) spend on research?

Principal Applicant	Coapplicant 1	Coapplicant 2	Coapplicant 3	Coapplicant 4
[**]	[**]	[**]		

(b) How many hours per week will be spent on this project by the Principal Applicant and Coapplicant(s)?

Principal Applicant	Coapplicant 1	Coapplicant 2	Coapplicant 3	Coapplicant 4
[**]	[**]	[**]		

Q8 RELATED APPLICATIONS

(a) Is this or a related application currently being submitted elsewhere? YES o NO x

If yes, to which organisation?

By what date is a decision expected? (dd/mm/yy)

(b) Has this, or a similar, application been submitted elsewhere over the past year? YES o NO x

If yes, to which organisation?

What was the result?

(c) Is this application a resubmission or has it been previously considered under another Wellcome Trust scheme? YES o NO x

If yes, when was it originally considered?

Please give the Wellcome Trust's reference number:

Briefly state how this application differs from the original (no more than 250 words)

Q9 SUMMARY OF PROPOSED RESEARCH INCLUDING KEY GOALS

(a) For scientifically qualified assessors: (no more than 200 words)

Elevated expression of Bmi-1, a stem cell polycomb protein (also called PCGF4) has been correlated with the chemo- and radio-resistance of a sub-fraction of tumour cells that have stem cell characteristics. These stem-like cells are thought to be responsible for tumour recurrence leading to treatment failures in many cancer types. Bmi-1 has been shown to play a significant role in many neoplasias. In particular, there is significant evidence that Bmi-1 overexpression in glioblastoma, including glioblastoma multiforme (GBM), is a key event for tumour growth. PTC has been searching for compounds that inhibit Bmi-1 protein expression and has identified low molecular weight compounds that potently and selectively target the post-transcriptional regulation of Bmi-1 expression to reduce the translation rate of Bmi-1 in cancer cells and in mouse xenograft models of glioblastoma. The top chemical series are in lead optimisation to improve their pharmaceutical properties. Our goal is to identify compounds that meet our criteria for a Development Candidate (DC). The research plan proposed in this application describes the drug development process of generating and optimizing chemical analogs to define structure-activity-relationships that improve efficacy, potency and pharmaceutical properties that would enable DC identification. Once identified, we will develop an acceptable drug formulation, initiate IND-enabling safety studies, and develop a process for large scale compound synthesis in preparation for a subsequent submission of an IND application.

(b) For lay readers: (no more than 200 words)

In this proposal we will be developing a novel drug that will target a protein called Bmi-1, a well established oncogene that has been shown to be overexpressed in tumour cells and necessary for cancer stem cell survival. By inhibiting Bmi-1 expression, we anticipate making resistant cancer

stem cells susceptible to treatment. Recent studies have also demonstrated that tumours have a sub-population of cells referred to as “cancer stem cells” that are involved in initiating tumour growth and progression. In addition, these stem-like cells are more resistant to chemical and radiation therapies than are other tumour cells. Thus, although a large portion of the tumour can be debulked by chemo- or radio-therapies, the stem-like resistant cells are a pool of cells that are resistant to cancer therapies, ultimately causing tumour recurrence. Thus, targeting Bmi-1 offers a strategy for therapeutic regimens intended to decrease treatment failures, thereby improving patient outcome.

Q10 What is the total requested cost from the Trust? — US$

Funding dependent milestones		Cumulative Costs	
Cost of Milestone Period 1 (M1):		$ 860,000	
Cost of Milestone Period 2 (M2):	$ 2,239,000	$ 3,099,000	=M1+M2
Cost of Milestone Period 3 (M3):	$ 2,298,000	$ 5,397,000	=M1+M2+M3

Q11 Define the primary objective of the proposal and briefly summarise the project work plan. (Maximum 1 page)

The primary objective of this program is the identification and subsequent development of a new class of a bioavailable small molecule that inhibits tumour growth by selectively reducing the Bmi-1 production, an important tumour stem cell factor. **Bmi-1 is amongst the most validated of stem cell-associated proteins, is overexpressed in a wide variety of cancers, and is shown to contribute to drug-resistance and treatment failure.** We plan to identify a development candidate (DC) for subsequent safety toxicology and clinical studies. We anticipate that this inhibitor will have activity either as monotherapy or in concert with standard-of-care cytotoxic agents to treat gliomas, and feasibly other cancers. As an epigenetic regulatory factor with no known enzymatic activity on which to base a functional assay, targeting of Bmi-1 requires an innovative approach as is proposed here.

PTC Therapeutics is a 12 year old Biopharmaceutical company that leverages its expertise in RNA biology to discover and develop small molecules that selectively modulate the expression of critical genes of medical importance. Using our RNA biology technologies, three compounds are currently in different stages of development. The goal of this proposal is to optimise and develop a low molecular weight inhibitor of Bmi-1 protein expression. **This program will be accomplished in three phases: Phase 1 and 2 encompass lead optimisation efforts which will improve the efficacy, safety and pharmaceutical properties of our lead scaffold in order to identify a Development Candidate. Phase 3 encompasses pre-clinical development work in support of the submission of an IND application.**

Using our GEMS™ technology to identify low molecular weight compounds that selectively modulate protein expression, we have performed a high throughput screen to identify compounds that reduce Bmi-1 expression, selected lead scaffolds, and performed preliminary structure-activity-relationship (SAR) studies. We have already been successful in identifying potent and selective molecules that reduce endogenous Bmi-1 protein expression, both in vitro and in vivo. Based on these results, our next steps will be to improve oral bioavailability and to determine the safety profile of the Development Candidate (DC). Specifically, the aims of this application are the following:

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 36 pages were omitted. [**]

Q19 Please describe the management of the project including the expertise and experience of the management team and the proposed Steering Committee membership

Leadership Team

Drs. Thomas Davis, Young-Choon Moon and Marla Weetall conceived and wrote this application through close cooperation and will serve as Co-applications for this project. Considering the complexity of this proposal, with its emphasis on the identification of a development candidate and development of the preclinical and nonclinical data packages sufficient for the submission of an IND application, we will leverage the diverse expertise and considerable qualifications of Drs. Davis, Moon and Weetall to ensure the successful completion of the aims of this proposal.

Dr. Thomas Davis has over 11 years experience in preclinical oncology drug development. He served as a Laboratory Director in the Department of Radiation Oncology of the Ireland Cancer Center of Case Western Reserve University, and participated in the development of radiosensitising compounds currently in clinical trials. Prior to joining PTC Therapeutics, he worked as a Principal Research Scientist and Group Leader performing preclinical studies of cyclo-oxygenase 2 inhibitors for oncology at Pharmacia/Pfizer. Additionally, at PTC Therapeutics, Dr. Davis has led the PTC299 preclinical oncology project.

Dr. Young-Choon Moon has over 25 years experience in Synthetic and Organic Chemistry, 15 years of which have been in drug discovery. During that time he has been involved in drug design, hit-to-lead optimization, medicinal chemistry, combinatorial chemistry, process chemistry, analytical chemistry, and project management at PTC Therapeutics, Vertex Pharmaceuticals, LG Life Science, and KRICT. Dr. Moon was the chemistry project leader for the discovery and development of PTC299, which is in Phase II trials for oncology. Additionally, he was a key contributor in the discovery and CMC development of ataluren (PTC124), which is in pivotal trials for Duchenne muscular dystrophy and cystic fibrosis.

Dr. Marla Weetall has over 16 years of industry drug discovery and development experience. While at PTC Therapeutics, she has established and manages the pharmacology and pharmaceutical profiling groups. Her teams have completed efficacy, non-GLP exploratory safety and DMPK studies resulting in the selection of four development compounds. Previously, Dr. Weetall participated in three programs that led to the selection of development candidates at Novartis.

Specific Responsibilities
[**].

Meetings and Data Exchange
[**]

[**].

Q20 OUTLINE OF PUBLIC ENGAGEMENT PLANS (max. ½ page)

As a company engaged in discovery and development of small molecule therapeutics for commercial purposes, PTC intends to maintain proprietary data from the research confidential for up to four years following the funding period.

During the four year period, PTC would expect to evaluate whether some or all of the data and related compound structures merit intellectual property protection.

Following this period, PTC would expect to share the data obtained from its research in the form of publications in the scientific literature at a suitable time.

As well, during the four year period, the Wellcome Trust may be free to publish or present on the data of the research pursuant to this grant, provided that the Wellcome Trust shall provide PTC with an advance copy of any proposed publication and text of any proposed presentation for review and comment at least [**] days in advance of its submission for publication or presentation. PTC shall have [**] days to review such advance copy. The Wellcome Trust agrees to delete any information that PTC deems to be confidential or that PTC requests be removed, and to delay any publication or presentation for an additional period of up to [**] additional days to permit the preparation and filing of patent application(s) in accordance with this grant. Any publication or presentation relating to research undertaken pursuant to the terms of this grant shall acknowledge PTC's contribution thereto in accordance with customary scientific practice.

In addition to data, a large proportion of the chemical structures involved in the research will become publicly available in the form of published patents. The availability of these chemical structures and related data to the public must not be prematurely disclosed to maintain and ensure the patentability of the compounds.

Further, PTC's policy has been to authorize only the publication of data related to the research in scientific journals and expect this to be the case for this program as well.

Please note that we provide support for researchers in the UK and Republic of Ireland to engage with the lay public. To receive information about training, funding and other public engagement opportunities, please tick the box. o

Q21 CURRICULUM VITAE OF APPLICANT(S)

This section should be completed by the Principal Applicant and all Coapplicants, can be duplicated if required.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of seven pages were omitted. [**]

Q22 BIOGRAPHIES OF ADVISORS AND STEERING GROUP

Neil Almstead, PhD, Senior Vice President, Research and CMC, PTC Therapeutics
Dr. Almstead joined PTC in 2000, and is responsible for PTC's efforts in research and manufacturing. Dr. Almstead joined PTC from Procter & Gamble (P&G) where he was directly involved in the areas of inflammatory diseases and oncology. At P&G he was project leader for both the Matrix Metalloproteinase and Map kinase inhibitor projects. Dr. Almstead has coauthored more than 75 publications and patents pertaining to the design and synthesis of lead candidate compounds for genetic disorders, oncology and inflammatory diseases. After receiving his Ph.D. in Organic Chemistry from the University of Illinois at Urbana-Champaign under the direction of Professor Scott Denmark, Dr. Almstead was a postdoctoral associate at the University of Basel in Switzerland with Professor Bernd Giese.

Joseph M. Colacino, PhD, Vice President, Drug Discovery, PTC Therapeutics
Dr. Colacino joined PTC in March 2003, and oversees all drug discovery activities across therapeutic areas. He brings over 14 years of drug discovery experience from Eli Lilly and Company. At Eli Lilly, he held positions as Research Acquisition Advisor, Director of Virology, and Head of Biology for Infectious Diseases Research. In that capacity, Dr. Colacino initiated and directed research programs in Infectious Diseases and managed a number of internal committees involved in the evaluation of screening methodologies and scientific approaches. Dr. Colacino is widely published and serves on the editorial boards of Antimicrobial Agents and Chemotherapy, Antiviral Chemistry and Chemotherapy, and Progress in Drug Research and has been an expert reviewer for Journal of Virology, Virology and Proceedings of the National Academy of Science. Dr. Colacino received his Ph.D. in Virology from the Cornell Graduate School of Medical Sciences, New York.

Eric N. Jacobsen, PhD, Sheldon Emery Professor of Organic Chemistry, Harvard University
Dr. Jacobsen is a member of PTC Therapeutics Scientific Advisory Board; in addition, he is a consultant for Merck & Co., Inc. and Amgen, Inc. Dr. Jacobsen has received numerous awards for his endeavors, including the NSF Presidential Young Investigator Award (1990), the Packard Fellowship (1991), the Camille and Henry Dreyfus Teacher-Scholar Award (1992), the Alfred P. Sloan Foundation Fellowship (1992), the Cope Scholar Award (1993), the Fluka "Reagent of the Year" Prize (1994), the Thieme-IUPAC Prize in Synthetic Organic Chemistry (1996), the Baekeland Medal (1999), the ACS Award for Creativity in Synthetic Organic Chemistry (2001), election to the American Academy of Arts & Sciences (2004), the ACS HC Brown Award for Creativity in Synthetic Methods (2008) and election to the National Academy of Sciences (2008). Dr. Jacobsen received his Ph.D. in Organometallic Chemistry from the University of California, Berkeley.

Dr. Harry Miao, MD, PhD, Director, Clinical Development, PTC Therapeutics
Originally receiving his MD in China at the University Medical College, Qingdao, Dr. Miao obtained his PhD from the Hebrew University, Hadassah Medical Center in Israel and performed a post-doctoral fellowship at Harvard Medical School. Dr. Miao's previous industry experience includes six years of work in angiogenesis research, tumour biology, and oncology drug safety at ImClone Systems before joining PTC in January 2007. Since coming to PTC, he has been central to development efforts for PTC299. He has provided the primary leadership role for multiple PTC299 clinical trials, including studies in patients with breast cancer, Kaposi sarcoma, neurofibromatosis, and other solid tumours. Harry has also been instrumental in generating new clinical trials for PTC299 in prostate cancer, glioblastoma, and pediatric brain tumours. Dr. Miao has also overseen the management of PTC's relationships with multiple contract research organizations.

Dr. Robert Schneider, PhD, Albert B. Sabin Endowed Chair for Molecular Pathogenesis, NYU School of Medicine
Dr. Schneider is a member of PTC Therapeutics Scientific Advisory Board, and holds a PhD in Biomedical Sciences from the Mt. Sinai School of Medicine. Dr. Schneider's research is directed to the development, progression and metastasis of breast cancer and the interplay of the inflammatory response. In addition, his research explores the molecular understanding of gene regulation by ionizing radiation, particularly its ability to regulate protein synthesis and use of this knowledge for development of new breast cancer treatment strategies. Dr. Schneider is an associate director of the NYU Cancer Institute, director of Translational Cancer Research, co-

Curriculum Vitae of Applicant(s)

director of Translational Research for the NYU Clinical Translational Research Institute, and co-director of the Breast Cancer Research Program at NYU School of Medicine.

Q23 PREVIOUS APPLICATIONS TO THE WELLCOME TRUST

(a) Is this the Principal Applicant's first application to the Wellcome Trust? YES x NO o

(b) Give details of all previous applications to the Wellcome Trust over the last five years. This information should be provided for the Principal Applicant and all Coapplicant(s). Please include name of grant holder, grant number (if known) and, if application was successful, the amount and period of award.

Q24 SUMMARY OF FINANCIAL SUPPORT REQUESTED - State currency used if not UK £ Sterling. **PLEASE NOTE: VAT ON CONTRACT RESEARCH AGREEMENTS IS ALLOWABLE. THESE AGREEMENTS ARE SUBJECT TO NORMAL PROCUREMENT PROCEDURES.**

Duration of grant (state in months): 30

	Total cost
(a) Salaries	[**]
(b) Materials and consumables	[**]
(c) Animals	[**]
(d) Equipment	[**]
(e) Miscellaneous	[**]
GRAND TOTAL	**5,397,000 US$**

Financial details

Q25 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED - State currency used if not UK £ Sterling.

(a) Salaries

Please refer to guidance notes and definition of terms for further details. Expand table as necessary. Budget is in US$

Post no.	Staff category	Name (if known)	Starting salary	Grade/ Scale	Increment date (dd/mm)	Start date (dd/mm/yy)	EFFORT ON PROJECT: Period on project (months)	EFFORT ON PROJECT: % of full time	London Allowance	Total of other allowances	Employer's contributions	Total cost on grant

Budget Continued							EFFORT ON PROJECT					
Post no.	Staff category	Name (if known)	Starting salary	Grade/ Scale	Increment date (dd/mm)	Start date (dd/mm/yy)	Period on project (months)	% of full time	London Allowance	Total of other allowances	Employer's contributions	Total cost on grant

Confidential Materials were omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted [**].

Q25 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)

Expand table as necessary.

(b) Materials and consumables (description)		Costs
Biology — Reagents		[**]
Cell/Tissue Culture		[**]
Assay Kits		[**]
Glassware		[**]
Cells		[**]
Chemistry — Reagents		[**]
Solvents		[**]
Glassware		[**]
Licenses		[**]
Misc		[**]
	Subtotal	**[**]**
(c) Animals		
Total purchase cost		[**]
Total maintenance cost		[**]
Total procedures cost		
	Subtotal	**[**]**

The table below should be duplicated for **each different species**.

(i)	**Animal species to be used, and strain if relevant**	[**]
		[**]
		[**]
		[**]
		[**]
(ii)	**Source of supply**	[**]
		[**]
(iii)	**Purchase**	
		[**]
		[**]
	Purchase price per animal	[**]
		[**]
		[**]
	Total number of animals to be purchased	[**]
		[**]
		[**]
	Total purchase cost	**[**]**
(iv)	**Maintenance**	

	Total number of animals to be maintained	[**]
		[**]
		[**]
		[**]
	Total number of weeks' maintenance required	[**]
		[**]
		[**]
		[**]
		[**]
	Cost per animal per week	[**]
		[**]
	Total maintenance cost	**[**]**
(v)	**Experimental procedures**	

Types of procedure(s)	
Cost per procedure(s)	
Total procedures cost	

Q25 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)
(d) Equipment

Please provide contact details for the Institution's Director of Procurement/Head of Purchasing (or equivalent).

Name: Tel:

Address: E-mail:

(i) Request for equipment. Expand table as necessary.

Type of equipment	Equipment specification	Preferred manufacturer/ supplier (if known)	Duration & total cost of maintenance contract to be purchased	Number of items	Cost per item	Total cost
					Total:	
					Contribution from other sources:	
					Amount requested:	

Q25 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)
(d) Equipment (cont.)

(ii) Request for equipment maintenance. Expand table as necessary.

Maintenance of existing Wellcome Trust-funded equipment

The Wellcome Trust will only consider providing maintenance funds for equipment more than five years old if the applicant can demonstrate it is cost-effective to do so.

Details of equipment/facility	Wellcome Trust grant reference number and start/end dates of original award	Date of purchase	Start/end dates of any current maintenance contract, length and total cost	% of time/hours of use for this project	Maintenance cost requested based on time used for this project

(iii) Request for access charges. Expand table as necessary.

Access charges

Details of equipment/facility	Original source of funding (provide Wellcome Trust grant reference number if applicable)	Standard access charge per hour/day	% of time/hours of use for this project	Access charge requested based on time used for this project

Q25 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.) — IN US$ Expand table as necessary.

(e) Miscellaneous (description)	Costs
Collaborations	[**]
CMC	[**]
IND Tox	[**]
Subtotal	**[**]**

Q26 ACCESS TO RADIATION SOURCES

(a) Will the proposed research require access to either the Synchrotron Radiation Source (SRS) at Daresbury or the European Synchrotron Radiation Facility (ESRF) at Grenoble? YES o NO x

If yes, please complete the table below, providing details of beam time requested and scheduling information (anticipated usage must be specified in whole days).

Synchrotron	Station	Special requirements (single bunch, other specify)	Total number of days	Number of days per annum				
				Year 1	Year 2	Year 3	Year 4	Year 5

(b) Please justify the stations and beam time requested (no more than 500 words).

(c) Will the proposed research require access to a neutron source? YES o NO o

If yes, complete Q26 (a) and (b) above indicating that it is a neutron source that is required, and Q25 (d)(iii) Access charges, detailing the costs required.

Q27 REASONS FOR SUPPORT REQUESTED

In this section, justify:

(a) Staff requested **specifying their roles, responsibilities and location, if appropriate,** with respect to the proposed project (no more than 700 words)

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of two pages were omitted [**].

Justification of costs requested

Q28 FULL ECONOMIC COSTING (UK applicants only)

The Wellcome Trust would like to monitor the full economic cost of research proposals. If your institution is calculating the full economic costs of this proposal, the table below should be completed.

Please note that the Wellcome Trust will not fund the full economic cost of research and the actual costs sought from the Wellcome Trust should be detailed in the 'DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED' section of the form.

This information is being gathered for monitoring purposes only and will have no bearing on the peer review and decision-making process for your application.

(a) Does the host institution use TRAC or an alternative methodology validated by the UK Research Councils to calculate full economic costs? YES o NO o

(b) If yes, please complete the following table:

	Full Economic Cost (£)	Contribution requested from the Wellcome Trust (£)
Directly Incurred Costs		
Staff		
Travel and subsistence		
Other costs		
Equipment		
Subtotal		
Directly Allocated Costs		
Principal Applicant salary costs		
Coapplicant salary costs		
Estates costs		
Other directly allocated costs		
Subtotal		
Indirect Costs		
TOTAL		

Administration

Q29 RESEARCH INVOLVING HUMAN PARTICIPANTS, BIOLOGICAL SAMPLES AND PERSONAL DATA

(a) Does your project involve human participants? YES o NO x
If yes, refer to notes.

(b) Will personal data be used? YES o NO x

(c)	Will your project involve use of biological samples?	YES o	NO x
(d)	Please state by whom the project will be, or has been, ethically reviewed, and specify any other regulatory approvals that have been, or will be, obtained.		
(e)	In the course of your project:		
(i)	Do you propose to use facilities within the National Health Service (NHS)?	YES o	NO x
(ii)	Does your research involve patients being cared for by the NHS?	YES o	NO x
(iii)	If the answer is yes to (i) or (ii) above, please indicate which organisation has agreed to be the sponsor for the project under the Research Governance Framework for Health and Social Care, published by the Department of Health in England or the corresponding departments in Northern Ireland, Scotland or Wales. Please note that the Wellcome Trust cannot act as sponsor.		
(f)	If your project involves a clinical trial:		
(i)	Please state whether it is covered by The Medicines for Human Use (Clinical Trials) Regulations.	YES o	NO o
(ii)	Please indicate which organisation has agreed to be the sponsor for the project. Please note that the Wellcome Trust cannot act as sponsor.		

Q30 EXPERIMENTS ON ANIMALS

(a)	Do your proposals involve the use of animals or animal tissue?	YES x	NO o
(b)	Do your proposals include procedures to be carried out on animals in the UK which require a Home Office licence? If yes, refer to notes.	YES o	NO x
(c)	Does the institution where the animal work is to be carried out hold a certificate of designation under the Animals (Scientific Procedures) Act 1986?	YES x	NO o
(d)	Do your proposals involve the use of animals or animal tissue outside the UK? If yes, refer to notes.	YES x	NO o
(e)	If your project does involve the use of animals, what would be the severity of the procedures?	Mild Moderate Substantial	x x x
(f)	Please provide details of any procedures of substantial or moderate severity (no more than 250 words). [**].		
(g)	Why is animal use necessary: are there any other possible approaches? (no more than 250 words)		

[**].

(h)	Why is the species to be used the most appropriate? (no more than 250 words) [**].		

Q31 RISKS OF RESEARCH MISUSE

(a)	It is the responsibility of institutions in receipt of Wellcome Trust funding to ensure that any risks that research could be misused for harmful purposes are managed in an appropriate manner. Please tick the box to confirm that you have considered whether your proposed research could generate outcomes that could be misused for harmful purposes.		x
(b)	If you have identified any tangible risks of this type, please briefly describe these risks and the steps that you and your institution will take to manage them (no more than 250 words). We have no indication that a oncology drug targeting the expression of the protein Bmi-1 would have potential for misuse.		

Q32 LOCATION OF RESEARCH

(a)	Will the research project be undertaken in a Wellcome Trust Clinical Research Facility?	YES o	NO x
	If yes, please specify:		

(b) Will the research project be undertaken in the Wellcome Trust Sanger Institute or a Wellcome Trust Centre? YES o NO x

If yes, please specify:

Please provide a letter of support from the Director of the Centre/Clinical Research Facility specified.

Q33 CONSULTANCIES AND EQUITIES

Do any of the applicants have consultancies or any equity holdings in companies or other organisations that might have an interest in the results of the proposed research? YES x NO o
If yes, refer to notes and give brief details (no more than 200 words).
As determined in his/her capacity as a PTC Therapeutics, Inc. employee

Q34 TREASURY POLICY

This scheme is managed through an agreement that includes the potential for advanced funding by the Wellcome Trust. The Trust requires an outline of your current 'treasury policy' prior to the release of funds. It is the Trust's normal policy that it will be willing to release advance funds where the treasury policy requires cash holdings to be held with banks of a Standard & Poor's AA- or higher rating, with the bank's headquarters being EU or US domiciled. Where the treasury policy includes other banks, this may still be acceptable for the purpose of the Trust releasing advance funds, but will be the subject of further review.

Please note, this will not have any impact on consideration on the merits of your application, but allows the Trust to ensure funding can be released in a timely manner, if your application is successful. Any changes in your treasury policy between application and award should be promptly notified to the Trust.

Wachovia Bank, N.A.

SUBJECT CLASSIFICATION

1. SYSTEMS AND PROCESSES

Choose one primary (compulsory) and up to three secondary (optional).

Drug and vaccine development
Cellular processes
Nervous system / Mental health

2. DISEASE

Choose one primary (compulsory) and up to three secondary (optional).

Cancer

3. DISCIPLINE

Choose one primary (compulsory) and up to three secondary (optional).

Health Services Research/Health Systems Research
Developmental biology
Pharmacology/Pharmacy

4.TECHNIQUE

Choose up to three (optional).

5. OTHER IDENTIFIER

Choose up to six (optional).

Translation/protein synthesis
Stem cells
Drug
Central nervous system

6. Tick all relevant boxes (compulsory).

BASIC	o
CLINICAL	x
TROPICAL	o
VETERINARY	o
TRANSLATION	x

COLLABORATION
ON A GRANT FORM

Reference Number:

Collaborators, i.e. scientific/medical colleagues, who are associated with a research proposal and named in the body of the application, but are not Coapplicants, are asked to complete this form.

Name of grant applicant:

Department and institution:

Name of collaborator:

Full address:

Title of research project:

Extent and nature of collaboration:
(A brief paragraph providing details of:

- The role and contribution of the collaborator, with an indication of the time the collaborator will spend on the project.
- Any reagents the collaborator will provide. Please indicate if there are any Intellectual Property issues or restrictions arising from Material Transfer Agreements.)

I confirm that I am willing to collaborate as stated above with on this research project

Signed: Date:

(if more than one copy of this form is required, duplicate as necessary)

Collaboration form

EQUAL OPPORTUNITIES
MONITORING FORM

CONFIDENTIAL

The Commission for Racial Equality and the Equal Opportunities Commission recommend collecting data to monitor the fairness of selection decisions. There is no obligation to provide this information but the Wellcome Trust would be grateful if the Principal Applicant would complete this form to assist with this process. On receipt, this form will be separated from the completed application form. The information provided will be regarded as strictly confidential and will be held on a secure database; it will not be shown to anybody involved in the processing of the application. The Wellcome Trust will anonymise these data (i.e. remove the applicant's name) when using them for statistical and research purposes.

Name:	[**]
1. Sex:	[**]
2. Date of birth:	[**]
3. Ethnic origin:	[**]
4. Disability:	[**]

Equal opportunities monitoring form

February 23, 2010

Thomas W. Davis, Ph.D.

Director/Oncology
PTC Therapeutics
100 Middlesex Business Center
South Plainfield, NJ 07080-2400

Dear Tom,

I am pleased to confirm my role as a collaborator on your Wellcome Trust grant. As you know we have developed and validated several systems for the isolation and characterization of tumor-initiating cells from a variety of cancers.

I'm enthusiastic about continuing our fruitful collaboration with PTC and look forward to evaluating PTC Therapeutics novel Bmi1 modulators in our systems. The potential of these inhibitors to be effective in multiple cancer types and the possibility of these compounds to inhibit tumor-initiating cells will have a major impact of cancer therapy.

I look forward to assisting you in these exciting studies and will help in anyway possible.

Sincerely,

Daniel J. Medina

Daniel J. Medina, Ph.D.
Associate Professor

195 Little Albany Street · New Brunswick, New Jersey 08903-2681 · Phone: 732.235.CINJ (2465) · www.cinj.org

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[ILLEGIBLE]

CONFIDENTIAL

Reference Number:

Collaborators, i.e. scientific/medical colleagues, who are associated with a research proposal and named in the body of the application, but are not Coapplicants, are asked to complete this form.

Name of grant applicant:	Thomas W. Davis, Ph.D.
Department and institution:	Oncology PTC Therapeutics, Inc.
Name of collaborator:	Daniel J. Medina
Full address:	The Cancer Institute of New Jersey 195 Little Albany Street New Brunswick, NJ 08903-2681
Title of research project:	[**]
Extent and nature of collaboration: (A brief paragraph providing details of:	[**]

- The role and contribution of the collaborator, with an indication of the time the collaborator will spend on the project.
- Any reagents the collaborator will provide. Please indicate if there are any Intellectual Property issues or restrictions arising from Material Transfer Agreements.)

I confirm that I am willing to collaborate as stated above with on this research project

Signed: Daniel J. Medina Date: 2/24/10

NewYork-Presbyterian
The University Hospital of Columbia and Cornell

Brain Tumor Center
The Neurological Institute
710 West 168th Street
New York, NY 10032
TEL 212 305 1718
FAX 212 305 1716

Steven S. Rosenfeld, M.D., Ph.D.
Co-director
Jeffery Bruce, M.D.
Co-director

February 24, 2010

Thomas W. Davis, Ph.D.

Director/Oncology
PTC Therapeutics
100 Middlesex Business Center
South Plainfield, NJ 07080-2400

Dear Tom:

It is a pleasure for me to acknowledge my enthusiastic support for your application to the Wellcome Trust for support of your proposed studies of Bmi1 inhibitors in glioblastoma. My laboratory has extensive experience in working with the retroviral models of glioblastoma that have been developed by my colleague, Dr. Peter Canoll (Department of Pathology, Columbia University), and we routinely generate mice with intracranial anaplastic gliomas with defined genetic lesions. These include EGFR amplification, EGFRvIII mutation, PTEN deletion, p53 deletion, both alone and in various combinations. These models are quite robust and reproduce all of the histologic features of human glioblastoma. The Neuro-Oncology Program at Columbia's Herbert Irving Comprehensive Cancer Center, of which I am Co-Director, also has the capability of utilizing non-invasive imaging to monitor the growth of these tumors, and includes both luminescence-based imaging as well as MRI. All of these capabilities will be available for your proposed studies.

As you know, there are no effective ways of targeting tumor stem cells in human glioblastoma, and our inability to affect this tumor cell population severely limits our ability to effectively treat this disease. I am thus very excited about your proposed studies and look forward to collaborating with you on this important project.

Sincerely,
/s/ Steven S. Rosenfeld

Steven S. Rosenfeld, MD, PhD
Professor and Co-Director, Neuro-Oncology Program
Herbert Irving Comprehensive Cancer Center
Columbia University
710 West 168th St.
Room 204
New York, NY 10032

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wellcome trust

EQUAL OPPORTUNITIES
MONITORING FORM
CONFIDENTIAL

Reference Number:

Collaborators, i.e. scientific/medical colleagues, who are associated with a research proposal and named in the body of the application, but are not Coapplicants, are asked to complete this form.

Name of grant applicant:	Thomas W. Davis, Ph.D.
Department and institution:	Oncology PTC Therapeutics, Inc.
Name of collaborator:	Steven Rosenfeld, MD, PhD
Full address:	710 West 168th St. New York, NY 10032
Title of research project:	
Extent and nature of collaboration: (A brief paragraph providing details of:	[**]

- The role and contribution of the collaborator, with an indication of the time the collaborator will spend on the project.
- Any reagents the collaborator will provide. Please indicate if there are any Intellectual Property issues or restrictions arising from Material Transfer Agreements.) [**]

I confirm that I am willing to collaborate as stated above with on this research project

Signed: [ILLEGIBLE] Date: 2/24/10

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted [**]

SCHEDULE 2

CONDITIONS

1. Compliance with PTCs obligations with respect to the formation and operation of the RSG in accordance with Clause 5;

2. Engagement and involvement of an independent industry adviser to the RSG with relevant experience in accordance with Clause 5.2(a)(ii);

3. Timely submissions of reports on Programme progress pursuant to Clause 5.7;

4. Co-operation with Site Visit Group prior to and during visits in accordance with Clause 6;

5. Timely consultation with the IPMG on patent strategy and patent prosecution in accordance with Clause 9.

6. Compliance with the Grant Conditions and any other agreements between PTC and the Trust relating to the Programme;

7. Seeking and maintaining protection of Background Intellectual Property in accordance with Clause 8.4; and

8. Compliance with PTC's treasury policy (such policy to be acceptable to the Trust) in acccordance with Clause 2.9.

SCHEDULE 3

MILESTONES, MILESTONE DATES AND TRANCHES

Milestone Number	Milestone Description	Milestone Date	Amount of Tranche
0	Signing of Agreement	Within [**] Business Days of the Commencement Date	$ [**]
1	[**] [**] [**]	[**] after Commencement Date*	$ [**]
2	[**] [**] [**]	[**] after Commencement Date*	$ [**]
3	[**] [**]	[**] after Commencement Date*	[**]

*** in accordance with the provisions of Clause 2.**

SCHEDULE 4

Costs of Goods

The Parties acknowledge and agree that in the event an unforeseen cost arises that is not specified below, they shall negotiate in good faith whether such cost is an allowable cost.

For the purpose of calculating the cost of Product a standard costing approach is to be applied in accordance with the Accounting Standard. The following types of expenses shall be included:

(i) direct materials;

(ii) direct labour;

(iii) indirect manufacturing costs;

(iv) quality assurance, and

(v) certain variances as set out in paragraph 5 below, but not including other Production costs, as identified below.

Each of these categories of expenses are further specified below.

In any event, the Cost of Goods shall include all cost elements appropriate under the Accounting Standard.

1. Direct Materials

Materials used in the manufacturing processes that are traced directly to the completed Product, such as:

· Inert raw materials or excipients

· Active substances/ingredients

- Packaging components such as bottles, caps, labels, etc.

2. Direct Labour

The cost of employees engaged in Production activities that are directly identifiable with Product costs. This shall exclude supervision, which is included in indirect labour, and Production support activities such as inspection, plant and equipment maintenance labour, and material handling personnel. Direct labour cost includes:

- Base pay, overtime, vacation and holidays, illness, personal time with pay and shift differential.
- Cost of employee fringe benefits such as health and life insurance, payroll taxes, welfare, pension and profit sharing.

3. Indirect Manufacturing Costs

Costs for plant and equipment are to be applied to standard costs taking normal capacity utilization as a reference.

Costs which are ultimately allocated to Product based on standard direct labour hours of the operating departments. These costs include:

- Indirect Production Labour - salaries of employees engaged in Production activities which are not classified as direct labour, including supervision, clerical, etc.
- Costs of Direct Labour - employees not utilized for the manufacturing of Product such as training and general duties.
- Indirect Materials - supplies and chemicals which are used in the manufacturing process and are not assigned to specific Products but are included in manufacturing overhead costs. Includes supplies for which direct assignment to Products is not practical.
- Utilities - expenses incurred for fuel, electricity and water in providing power for Production and other plant equipment and waste disposal.
- Maintenance and Repairs - amount of expense incurred in-house or purchased to provide services for plant maintenance and repairs of facilities and equipment.
- Other Services - purchased outside services and rentals such as the cost of security, ground maintenance, etc.
- Depreciation - of plant and equipment utilizing the straight-line method of calculation.
- Insurance - cost of comprehensive and other insurance necessary for the safeguard of manufacturing plant and equipment.
- Taxes - expense incurred for taxes on real and personal property (manufacturing site, buildings and the fixed assets of equipment, furniture and fixtures, etc.) If manufacturing site includes other operations (marketing, R&D, etc.), taxes are allocated to manufacturing on the basis of total real and personal property.
- Cost of manufacturing, service departments - such as (where applicable):
  - Packaging Engineering
  - Manufacturing Maintenance
  - Industrial Engineering
  - Receiving and Warehousing
  - Purchasing and Accounting

  - Production Scheduling
  - Inventory Management
  - Plant Materials Management
  - Central Weigh
  - Manufacturing Administration
- Allocated costs of services provided to manufacturing including: (where applicable):
  - Cafeteria
  - Personnel Operations

- Health and Safety Services
- Division Engineering and Operations Services
- Plant Services (housekeeping)
- Manufacturing Information Systems
- Plant Power
- Office of V.P. Manufacturing

Various bases are used for allocating these costs to manufacturing operating departments including headcount, square feet, metered utilities use, estimated services rendered, EDP computer hours, etc.

4. Quality Assurance Costs

Direct labour and indirect costs for quality assurance departments testing and approving materials used in manufacturing and completed manufacturing batches and finished Products. This includes all manufacturing in-process testing and testing of finished materials. Excluded from Product costs are quality assurance costs related to research and development, stability testing, and other costs customarily excluded from such quality assurance costs.

5. Variance Costs

- Standard Cost of Goods include cost elements which are set at so-called standard costs. They serve as a norm on how much typically a Product costs. Deviations from such standard costs are captured in variances
- Inventory re/devaluation shall mean the gain or loss as a result of the inventory value adjustment due to changes in the standard costs.

- Non-Product related Production costs shall contain technical operations corporate headquarter overhead costs, non Product allocated QA costs, validation costs, directly expensed IT Programme costs, and other costs that cannot be attributed to specific Products.
- Warehousing & distribution costs are costs related to warehousing and distribution activities for finished goods to be shipped to 3rd parties.
- Write-offs are captured for the destruction of Products that cannot be used anymore due to expiration of shelf-life, spoilage in the Production process, and transportation mishaps.
- Third Party royalties are manufacturing and/or supply royalties paid to Third Parties

The following expenses are not included in Production costs:

a) Inventory carrying costs;

b) Regulatory affairs costs;

c) Pilot plant costs, research batches and other similar costs prior to turnover to manufacturing (excluding commercial goods produced by a research facility);

d) Costs incurred by manufacturing for special Programmes to establish and certify new Production processes, batch sizes and Product line improvements, and new vendor certification of equipment and primary materials components;

e) Manufacturing start-up costs and initial one-time extraordinary manufacturing costs incurred prior to plant operation and achievement of a normal Production activity level; including the costs of training, testing, qualification/validation of new equipment and facilities and initial, trial batches;

f) Significant idle capacity is eliminated from factory overhead and Product cost;

g) Finished goods warehousing, shipping and other distribution costs;

h) Product liability and/or business interruption insurance expenses; and

i) Intercompany profit.

SCHEDULE 5

In-kind contributions from PTC and the PTC Group

1. Infrastructure: laboratories and office space, including the costs of buildings, rental, fixtures and utilities) at the PTC's facilities in New Jersey, USA (the "**PTC Facilities**") and provision of the services of the PTC FTEs to the extent set out in the Application.

2. Access to existing equipment and laboratory benches within the PTC Facilities.

SCHEDULE 6

REVENUE SHARING TERMS

1) Introduction

a) This Schedule 6 sets out the revenue sharing terms ("Revenue Sharing Terms") agreed between the Parties.

b) Each scenario below shall apply based on the description of the scenario.

2) **Scenario 1**: PTC exploits the Programme Intellectual Property on a For-Profit Basis alone (or in collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of worldwide commercialization).

a) PTC shall pay the following stage-based milestones based on multiples of the total Trust Contribution through Regulatory Approval:

i) Milestone triggering events and amounts:

[**]

ii) Worked Example: assumes Trust funds $5.4 million US and PTC does not elect to defer payment of the Phase 3 milestone:

(1) Phase 1 milestone amount = $[**]

(2) Phase 2 milestone amount = $[**]

(3) Phase 3 milestone amount = $[**]

(4) Regulatory Approval milestone amount = $[**]

(5) Total of all milestone amounts= $[**]

iii) Worked Example: assumes Trust funds $5.4 million and PTC defers at Phase 3:

(1) Phase 1 milestone amount = $[**]

(2) Phase 2 milestone amount = $[**]

(3) Phase 3 milestone amount = $[**]

(4) Regulatory Approval milestone amount = $[**]

(5) Total of all milestone amounts = $35,640,000

iv) Payment of milestones

(1) Phase 1-Phase 3 milestones shall be payable in equal quarterly installments over expected term of study, with the first installment payment due within [**] Business Days of the milestone triggering event. PTC may by written notice to the Trust elect to defer payment of the Phase 3 milestone until after completion of the first Phase 3 study to be completed in either the USA or the EAA (whichever is the sooner) required for Regulatory Approval (the "Trigger Phase 3"), in which case PTC shall pay such Phase 3 milestone (including additional 1x due for the deferral option) within [**] of completion of the Trigger Phase 3 study. For clarity, if deferred, the payment of Phase 3 milestone is due regardless of outcome of Phase 3 trial(s); provided, that the Trust agrees to accept alternative consideration, such as equity, in the event a cash payment after a Phase 3 trial failure would place PTC in financial distress.

(2) For clarity, milestones are payable only for the first Product to reach the applicable milestone.

(3) The Regulatory Approval milestone shall be payable on the [**]; provided, however, that the Trust will consider in good faith payment of the Regulatory Approval milestone in installments if PTC revenue from all products at the time of Regulatory Approval is less than $[**].

b) In addition to any milestones payable in accordance with the preceding section, PTC shall also pay royalties on Net Sales of Products, on a Product-by-Product basis; provided, that such royalties shall only be payable in the event the Trust Contribution represents at least [**]% of the proposed $5.4 million US funding amount, and shall be scaled proportionately in the event the Trust Contribution is greater than [**]% but less than 100% of the proposed $5.4 million US funding amount:

i) Royalty scale based on Net Sales of Product:

(1) First $[**]%

(2) Next $[**]%

(3) Next $[**]%

(4) Next $[**]%

(5) Over $[**]%

ii) Royalties payable shall be payable on a country-by-country basis until the longer of (a) the expiration last Valid Claim of a patent in the applicable country or region covering the Product, or (ii) the expiration of marketing exclusivity of a Product in the applicable country or region based on applicable law.

3) **Scenario 2**: PTC exploits the Programme Intellectual Property on a For-Profit Basis through outlicensing of a Product to a Third Party on a worldwide, exclusive basis prior to Regulatory Approval.

a) The parties shall hold an economic stake ("Base Shares") in the Product calculated as of outlicensing effective date based on their respective economic contributions.

i) On the Commencement Date, PTC begins with $5.4 million Base Shares, and the Trust with zero.

ii) As the Trust pays the proposed the proposed $5.4 million US funding amount over the Programme Term, the Trust's Base Share shall increase proportionately. By way of example, [**].

iii) Following the Programme Term, PTC's ownership of Base Shares shall increase proportionately based on PTC's continuing economic contribution. By way of example, [**].

b) All consideration attributable to outlicensing to a Third Party (other than debt at arm's length interest rates or bona fide research funding) shall be divided between PTC and the Trust according to relative Base Share ownership at the time of such outlicensing. By way of example, [**].

c) For clarity, once outlicensing under this scenario has occurred, then the milestones provided for in scenario 1 shall no longer apply following the effective date of the outlicense; provided, that if a milestone trigger event occurred prior to the outlicense but installment payments are ongoing, PTC must complete such milestone payments.

d) For clarity, neither PTC nor the Third Party gaining the outlicense shall make any royalty payments to the Trust under this scenario.

e) License or access payments to Third Parties for enabling technologies required, in the good faith judgment of PTC, to develop and commercialize a Product shall be counted in the calculation of Base Shares under this scenario; provided, however, that such payments shall not include license or access payments made with respect to the composition of matter or method of use of those active ingredient(s) in the Product that incorporate, comprise or are derived from the Programme Intellectual Property.

4) **Scenario 3**: PTC exploits the Programme Intellectual Property on a For-Profit Basis by retaining development/commercialization rights to Product in some regions of the World or with respect to some uses of the Product (either alone or in a collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of commercialization)), and outlicenses the Product on an exclusive basis in other regions of the World or with respect to other uses of the Product.

a) In this scenario, any consideration from outlicensing (other than debt at arm's length interest rates or bona fide research funding) shall be divided between the parties according to Base Shares as of effective date of the outlicense.

b) In addition, following such outlicense, PTC shall pay milestones and royalties based on scenario 1 for those regions of the World or uses of the Product for which it retains rights, subject to the following adjustments:

i) PTC will prepare a written proposal for adjustment to milestones and royalties based on its modeling of the relative values of market share outlicensed vs. market share retained by PTC.

(1) The Trust shall consider PTC's proposal in good faith, and prepare a written counterproposal if it wishes;

(2) The parties shall negotiate in good faith for reasonable allocation of relative value of markets based on their proposals;

(3) If the parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

(4) Once the relative value of the markets outlicensed versus the markets retained by PTC is determined, PTC's obligation to make continuing milestone and royalty payments pursuant to Scenario 1 shall be reduced according to relative value of markets outlicensed versus the markets retained. By way of example, if PTC outlicensed [**] of the market value of a Product, then a milestone payment of $[**] owed under scenario 1 would be reduced to a milestone payment of $[**] under this scenario 3, and a [**]% Net Sales royalty under scenario 1 would become a [**]% Net Sales royalty under this scenario 3.

5) **Other Scenarios:**

a) If a situation arises that is not covered by any of the foregoing three scenarios, the parties will negotiate in good faith for an appropriate economic arrangement based on Base Shares.

i) If the parties cannot agree, each party shall prepare a written proposal and accompanying rationale for an appropriate economic arrangement.

ii) The parties shall then negotiate in good faith based on their respective proposals.

iii) If parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

Signed for and on behalf of

PTC THERAPEUTICS, INC.

by its duly authorised representative:

Signature: /s/ Stuart W. Peltz

Name: Stuart W. Peltz

Title: President & CEO

Date: May 26, 2010

Signed for and on behalf of

THE WELLCOME TRUST LIMITED as trustee of the Wellcome Trust

by its duly authorised representative:

Signature: /s/ Richard Seabrook

Name: Dr Richard Seabrook

Title: Head of Business Development Technology Transfer

Date: 26/May/2010

Signed for and on behalf of

THE WELLCOME TRUST LIMITED as trustee of the Wellcome Trust

by its duly authorised representative:

Signature: /s/ Bina Rawal

Name: Dr Bina Rawal

Title: Head of Medical Affairs Technology Transfer

Date: 26/5/10

Exhibit 10.17

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

DATED 21 Dec 2011

(1) THE WELLCOME TRUST LIMITED

and

(2) PTC THERAPEUTICS, INC.

AGREEMENT FOR THE PROVISION OF FUNDING TO PTC THERAPEUTICS, INC. FOR RESEARCH RELATING TO THE LEAD OPTIMISATION AND DEVELOPMENT OF NOVEL BACTERIAL DNA SYNTHESIS INHIBITORS FOR THE TREATMENT OF NOSOCOMIAL INFECTIONS CAUSED BY MULTI-DRUG RESISTANT GRAM-NEGATIVE BACTERIA

Execution Version

THIS AGREEMENT is made and entered into as of the day of December 2011 (the “**Commencement Date**”)

BETWEEN:

(1) **THE WELLCOME TRUST LIMITED** a company registered in England under number 2711000 as Trustee of the Wellcome Trust, a charity registered in England under number 210183, whose registered office is at 215 Euston Road, London NW1 2BE (the “**Trust**”); and

(2) **PTC THERAPEUTICS, INC.** a company incorporated and registered in the State of Delaware whose principal place of business is at 100 Corporate Court, South Plainfield, NJ 07080-2449, USA (“**PTC**”).

RECITALS:

(A) PTC is undertaking a research programme aimed at the lead optimisation and development of novel bacterial DNA synthesis inhibitors for the treatment of nosocomial infections caused by multi-drug resistant Gram-negative bacteria;

(B) In order to further its charitable objectives, the Trust is willing to make a funding award (the "**Award**") to PTC under the Trust's Seeding Drug Discovery Strategic Award Programme to enable PTC to undertake the research programme set out in the Application (as defined below) in accordance with the provisions of this Agreement.

1. **INTERPRETATION**

1.1 In this Agreement, unless the context otherwise requires:

"Accounting Standard"	means in the case of PTC and its Affiliates, US GAAP (United States Generally Accepted Accounting Principles), and in the case of Trust and its Affiliates, IFRS (International Financial Reporting Standards), in either case as generally and consistently applied throughout each Party's organisation, provided that PTC and its Affiliates may elect to convert to IFRS at any time on an organisation-wide basis;
"Affiliate"	means, with respect to a given entity, any person, corporation, partnership or other entity, that Controls, is Controlled by, or is under common Control with such entity;
"Agreement"	means this agreement;
"Application"	means the application made by PTC to the Trust for an award as set out at Schedule 1 as amended by Schedule 8 of this Agreement;
"Auditor"	shall have the meaning given to it in Clause 14;
"Award"	shall have the meaning given to it in the Recitals;

"Award Amount"	means an award of up to five million and thirty three thousand United States dollars (US$5,033,000);
"Background Intellectual Property"	means: (a) any Intellectual Property created, devised, generated, owned by or licensed to the PTC Group or which the PTC Group has rights to prior to the Commencement Date (but excluding, for the avoidance of doubt, the Programme Intellectual Property), which is necessary or useful for undertaking the Programme, for the protection or exploitation of the Programme Intellectual Property, and/or which is necessary or useful for the development and exploitation of the Programme Intellectual Property; and (b) The following provisional patent applications : [**]
"Background Know-How"	Means any and all Know-How and any components thereof (including without limitation manufacturing processes and quality control procedures) which are used by the PTC Group at any time for the Programme which are necessary or useful for undertaking the Programme, for the protection or exploitation of the Programme Intellectual Property, and/or which are necessary or useful for the development and exploitation of the Programme Intellectual Property, but excluding the Programme Intellectual Property. For the avoidance of doubt, PTC Background Know-How shall include Know-How generated by any PTC Affiliate and subsequently used by PTC for the Programme;
"Base Shares"	shall have the meaning given to it in Schedule 6;
"Books"	shall have the meaning given to it in Clause 14;
"Business Day"	means a day on which banks are normally open for business and which is not a Saturday or Sunday or a bank or public holiday in England, or in the State of New Jersey, USA;
"Change of Control"	means the acquisition by any Third Party of Control of PTC, other than (a) acquisitions by employee benefit plans sponsored or maintained by PTC, (b) the initial public offering of PTC, (c) the acquisition by an institutional investor (or group of institutional investors), such as a venture capital fund, private equity fund or hedge fund, of shares of PTC for

	investment purposes in a transaction approved by PTC's Board of Directors, or (d) a business combination involving PTC pursuant to which the stockholders of PTC immediately prior to such business combination beneficially own directly or indirectly more than fifty percent (50%) of the then outstanding common shares or voting power of the entity resulting from such business combination;
"Clinical Trial"	means a clinical trial conducted in accordance with recognised protocols approved by a Competent Authority;

"Co-applicant" means Dr. Arthur Branstrom of PTC;

"Commencement Date" means the date of this Agreement as set out at the top of page 3;

"Competent Authority" means any local or national agency, authority, department, inspectorate, minister, ministry official or public or statutory person (whether autonomous or not) of, or of any government of, any country having jurisdiction over this Agreement or any of the Parties or over the development or marketing of drugs including the European Commission and the European Court of Justice, the US Food and Drug Administration, the European Medicines Agency (or any successor entity) and any national regulatory authorities;

"Conditions" means the conditions set out at Schedule 2 which must be satisfied (to the reasonable satisfaction of the Trust) at all times during the Programme;

"Confidential Information" means any and all data, results, Know-How, show how, software, plans, details of research work, discoveries, inventions, intended publications, intended or pending patent applications, designs, technical information, business plans, budgets and strategies, business or financial information or other information in any medium and in any form, and any physical items, prototypes, compounds, samples, components or other articles or Materials disclosed on or after the Commencement Date of this Agreement by one Party to another Party whether orally or in writing or in any other form including for the avoidance of doubt Background Intellectual Property and Background Know-How;

"Control" means, in relation to PTC, where a person (or persons acting in concert) directly or indirectly including through any subsidiary or holding company or subsidiary of such holding company:

(a) has beneficial ownership over more than fifty

per cent (50%) of the total voting rights conferred by all the issued shares in the capital of the company which are ordinarily exercisable in general meeting; or

(b) has the right to appoint or remove a majority of its directors; or

(c) has power to direct that the affairs of the company are conducted in accordance with its wishes;

in each case where such person or persons did not have such beneficial ownership, right or power at the Commencement Date;

and "**Controlled**" and "**Controlling**" shall be construed accordingly;

"Cost of Goods" means in respect of any Products the fully allocated cost of manufacture as calculated in accordance with the accounting standard applicable to the selling party, consistently applied in accordance with Schedule 4 together with any and all royalties payable to any Third Party for technology directly related to the supply of the Products including without limitation any formulation technology and access to Materials owned by Third Parties;

"Development" means research and development activities relating to the Programme Intellectual Property and any Products following completion of the Programme including Clinical Trials and "**Develop**" shall be construed accordingly;

"Disclosure Letter" means:

(a) as at the Commencement Date, the disclosure letter dated the same date as this Agreement and accepted by the Trust; and

(b) after the Commencement Date, the disclosure letter as subsequently amended and agreed by the Parties immediately prior to the payment of each Tranche (or first installment of the each Tranche) of the Award Amount;

"Distributor" means a Third Party with whom PTC enters into a standard commercial distribution or sales arrangement with respect to marketing or sales of Product in a particular territory or region. For clarity, although PTC may grant a licence to a Distributor in support of such arrangement, a Distributor shall not constitute a Licencee for the purposes of this

Agreement;

"Documents" means reports, research notes, charts, graphs, comments, computations, analyses, recordings, photographs, paper, notebooks, books, files, ledgers, records, tapes, discs, diskettes, CD-ROMs, computer programs and documents thereof, computer information storage means, samples of material, other graphic or written data and any other media on which Know-How can be permanently stored;

"End of Award Report" shall have the meaning given to it in Clause 2.5;

"Expert" shall have the meaning given to it in Clause 19;

"Exploit"	means exploitation activities after completion of the Programme including obtaining Marketing Approval and the commercialisation, licensing, marketing, distribution and sales of Programme Intellectual Property and any Products utilising the Programme Intellectual Property including on a Not-for-Profit Basis and "**Exploited**" and "**Exploitation**" shall be construed accordingly;
"Exploiting Party"	means the Party or Parties undertaking development and exploitation pursuant to Clauses 11 and 12;
"Field"	means the use of small molecules that inhibit bacterial DNA synthesis using targets including DNA gyrase and/or topoisomerase IV for the treatment, mitigation, diagnosis or prevention of diseases in man or other animals;
"For Profit Basis"	means sales of Products on other than a Not For Profit Basis;
"FTE"	means one full time equivalent employee based upon a total of [**] hours worked per annum, not including time off; provided, that employees shall be accounted for on a percentage of effort basis;
"Further Funding"	means research funding support from a Third Party obtained in accordance with Clause 2.16, where such Third Party takes no benefit (whether financial, equity or in kind), for example US government funding;
"Intellectual Property"	means: (a) patents, designs, trade marks and trade names (whether registered or unregistered), copyright and related rights, database rights, Know-How and confidential information; (b) all other Intellectual Property and similar or

	equivalent rights anywhere in the world which currently exist or are recognised in the future; and (c) applications, extensions and renewals in relation to any such rights;
"Invention Policy"	shall have the meaning given to it in Clause 4.3;
"IPMG"	means the Intellectual Property Management Group established in accordance with Clause 9;
"IPMG Member"	means a member of the IPMG;
"Know-How"	means any technical and other information which is not in the public domain, including information comprising or relating to concepts, discoveries, data, designs, formulae, ideas, inventions, methods, models, assays, research plans, procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development), processes (including manufacturing processes, specifications and techniques), laboratory records, chemical, pharmacological, toxicological, clinical, analytical and quality control data, trial data, case report forms, data analyses, reports, manufacturing data or summaries and information contained in submissions to and information from ethical committees and regulatory authorities and computer programs or algorithms. Know-How includes Documents containing Know-How, including but not limited to any rights including trade secrets, copyright, database or design rights protecting such Know-How. The fact that an item is known to the public shall not be taken to preclude the possibility that a compilation including the item, and/or a development relating to the item, is not known to the public;
"Lead Compound"	means any Programme Compound which satisfies the criteria set out in Schedule 8 of this Agreement for use in the Field;
"Licencee"	means a Third Party other than a Distributor to whom PTC grants a license to Exploit Programme Intellectual Property in the Field in a *bona fide*, arms-length transaction.
"Major Market"	means any of the United States, the United Kingdom, Japan, and any two of the following: France, Spain, Germany and Italy.
"Marketing Approval"	mean all approvals, licences, registrations or authorisations of any federal, state or local regulatory agency, department, bureau or other governmental

	entity, necessary for the marketing and sale of Products in a regulatory jurisdiction (including in the case of countries where no national agency exists for the approval of small molecules, approval by the World Health Organisation);
"Material"	means any chemical or biological substance including any: (a) organic or inorganic element; (b) nucleotide or nucleotide sequence including DNA and RNA sequences;

(c) gene;

(d) vector or construct including plasmids, phages or viruses;

(e) host organism including bacteria, fungi, algae, protozoa and hybridomas;

(f) eukaryotic or prokaryotic cell line or expression system or any development strain or Product of that cell line or expression system;

(g) protein including any peptide or amino acid sequence, enzyme, antibody or protein conferring targeting properties and any fragment of a protein or a peptide enzyme or antibody;

(h) drug or pro-drug;

(i) assay or reagent;

(j) any other genetic or biological material or micro-organism; data for the derivation of molecular structures including NMR spectra, X Ray diffraction patterns and other primary experimental information, assignments and other calculations required for determination of the structure, and co-ordinates of the derived molecular structure; and

(k) transgenic animals;

"Milestones" means the Milestones as described in Schedule 3, and "**Milestone**" means any one of them;

"Milestone Dates" means the dates set out in Schedule 3 for the achievement of a Milestone and "**Milestone Date**" means any one of them;

"Milestone Extension" shall have the meaning given to it in Clause 2.4;

"Milestone Report" shall have the meaning given to it in Clause 2.3;

"Net Sales" means the net sales on behalf of PTC and any of its Affiliates or Distributors for the Products sold to Third Parties other than Licensees, as determined in accordance with PTC's usual and customary accounting methods, which are in accordance with the Accounting Standards.

(a) In the case of any sale or other disposal of a Product between or among PTC and its Affiliates or Distributors, for resale, Net Sales shall be calculated only on the value charged or invoiced on the first arm's-length sale thereafter to a Third Party;

(b) In the case of any sale which is not invoiced or is delivered before invoice, Net Sales shall be calculated at the time of shipment or when the Product is paid for, if paid for before shipment or invoice;

(c) In the case of any sale or other disposal for value, such as barter or counter-trade, of any Product, or part thereof, other than in an arm's length transaction exclusively for money, Net Sales shall be calculated on the value of the non-cash consideration received or the fair market price (if higher) of the Product in the country of sale or disposal; and

(d) In the event the Product is sold as a Combination Product, the Net Sales of the Product, for the purposes of determining royalty payments, shall be determined by multiplying the Net Sales of the Combination Product by the fraction, A/(A+B) where A is the weighted (by weight, if the Product is priced by weight; otherwise, by patient dose,) average sale price in a particular country of the Product when sold separately in finished form and B is the weighted average sale price in that country of the other product(s) sold separately in finished form. In the event that such average sale price cannot be determined for both the Product and the other product(s) in combination, PTC shall in good faith propose a relative allocation of value (and supporting methodology) for determining Net Sales for purposes of royalty payments with respect to such Combination Product, and Trust shall consider such proposal in good faith, and the Parties shall seek to reach agreement on such

allocation. If the Parties are unable to reach such agreement within [**] days after PTC provides such proposal, the issue shall be referred for binding resolution to a mutually agreeable individual (not affiliated with either Party) with expertise in the marketing and sales of similar pharmaceutical products similar to the Combination Product at issue (including experience in pricing and reimbursement), such resolution to occur within [**] Business Days after such referral.

"Non-Exploiting Party" means the Party or Parties not undertaking Development and Exploitation;

"Not for Profit Basis" means (a) sales of Products where the consideration received by the seller is less than or equal to the sum of the Cost of Goods for such Product; in calculating the consideration received by the seller account shall be taken of any

equity or lump sum or other payments received by the seller in respect of the Product; or (b) or sales of Products where the seller is a charitable organisation (other than the Trust or its Affiliates) under applicable law;

"Parties" means the parties to this Agreement, or any of them, as the context may require, and "**Party**" shall be interpreted accordingly;

"Phase 1 Clinical Trial " means a human clinical trial in any country, the principal purpose of which is a preliminary determination of safety in individuals or patients, that would satisfy the requirements of 21 C.F.R. §312.21(a), or an equivalent clinical study required by a Competent Authority outside the United States.

"Phase 2 Clinical Trial " means a human clinical trial conducted in any country, intended to explore multiple doses, dose response or duration of effect to generate initial evidence of safety and activity in a target patient population, that would satisfy the requirements of 21 C.F.R. §312.21(b), or an equivalent clinical study required by a Competent Authority outside the United States.

"Phase 3 Clinical Trial " means a human clinical trial in any country that would satisfy the requirements of 21 C.F.R. §312.21(c), or an equivalent clinical study required by a Competent Authority outside the United States that is prospectively designed to confirm with statistical significance in an expanded patient population the efficacy and safety of a drug in a given patient population, and the results of which are intended, alone or in combination with any other Clinical Trial, to form the basis for Marketing Approval by a

Competent Authority;

"PTC Group" means PTC and any Affiliate of PTC;

"PTC Previous Research" PTC's research, prior to the Commencement Date, in a programme aimed at the discovery, lead optimisation and development of novel inhibitors for the treatment of bacterial infections and funded in part by the U.S. Government;

"PTC TPC" means PTC's Treasury Policy Contact as set out at Clause 2.9 or such other contact as may be notified by the Company to the Trust in writing from time to time in accordance with Clause 2.9;

"Policies and Positions" means the policies and positions of the Trust for grants from time to time, which are set out at http://www.wellcome.ac.uk/node3610.html;

"Principal Investigator" means Dr. Vara Prasad V.N. Josyula of PTC;

"Product" means any Product developed by any member of the PTC Group or any Third Party incorporating, comprising or derived from the Programme Intellectual Property in finished dosage pharmaceutical form;

"Programme" means the research and development programme described in the Application and funded by the Trust pursuant to the Award and the terms of this Agreement; provided, that the RSG may amend the research and development programme from time to time in accordance with this Agreement;

"Programme Compound" means any compound identified or designed by PTC or another member of the PTC Group following the Commencement Date in respect of which activities are undertaken by (or on behalf of) PTC in the course of the Programme, and in each case shall include the chemical compound as well as esters, salts, hydrates, solvates, polymorphs and isomers thereof;

"Programme Books and Records" shall have the meaning given to it in Clause 4.4;

"Programme Intellectual Property" means any Intellectual Property (including the Programme Patents) created, devised or arising out of PTC or Staff undertaking and performance of the Programme or any part of it, including, without limitation, the Lead Compounds;

"Programme Inventions" means any inventions created, devised or arising out of PTC or Staff undertaking and performing the

Programme or any part of it;

"Programme Patents" means any patent applications that may be made by a member of the PTC Group or by the Trust on behalf of a member of the PTC Group (as appropriate) which claim any Programme Inventions or parts thereof, and any patents resulting from any such applications, utility certificates, improvement patents and models and certificates of addition and all foreign counterparts of them in all countries, including any divisional applications and patents, refiling, renewals, continuations, continuations-in-part, patents of addition, extensions (including patent term extensions), reissues, substitutions, confirmations, registrations, re validations, pipeline and administrative protections and additions, and any equivalents of the foregoing in any and all countries of or to any of them, as well as any supplementary protection certificates and equivalent protection rights in respect of any of them;

"Programme Term" means the time period commencing on the Commencement Date and ending on the earlier of completion of the Programme or three (3) years;

"PubMed Central"	means an archive of life science journal literature operated by the National Center for Biotechnology Information, a division of the US National Library of Medicine accessible at http://www.pubmedcentral.nih.gov/;
"Quarter"	means a period of three (3) consecutive calendar months commencing on 1 January, 1 April, 1 July or 1 October in any year and "**Quarterly**" shall be construed accordingly;
"Research Steering Committee" and "RSG"	means the group of persons constituted in accordance with Clause 5;
"Revenue Sharing Terms"	shall have the meaning given to it in Schedule 6;
"Site Visit Group"	means the group constituted in accordance with Clause 6;
"Staff"	means all scientific and technical staff, who are employees, students, officers, contractors, independent consultants or otherwise of PTC (or any other member of the PTC Group) and who participate in the Programme including without limitation the Principal Investigator and the Co-applicant, together with any relevant administrative staff assisting with the Programme;

"Third Party"	means any entity or person other than the Parties or an Affiliate of a Party;
"Tranches"	means the tranches of the Award Amount payable by the Trust to PTC as set out in Schedule 3, and "**Tranche**" shall mean each of them;
"Treasury Policy"	means PTC's treasury policy as amended from time to time. A copy of PTC's treasury policy (in English) as at the Commencement Date is set out at Schedule 7;
"Trust Contribution"	Means (a) tranches of the Award Amount paid by the Trust to PTC and (b) in the event a Milestone has been completed but PTC (i) has failed to submit a Milestone Report or request payment of the next tranche of the Award Amount, or (ii) is otherwise in breach of the Agreement and such breach would give rise to a termination right on the part of the Trust pursuant to Clause 20.2 or 20.3, those tranches of the Award Amount that would have been payable by the Trust to PTC but for PTC's omission or breach;
"Trust TPC"	means the Trust's Treasury Policy contact as set out at Clause 2.10 or such other contact as may be notified by the Trust to the Company in writing from time to time in accordance with Clause 2.10;
"Valid Claim"	shall mean a claim of an issued Programme Patent, or a claim of a pending patent application or a supplementary protection certificate of a Programme Patent that has not expired or been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment; provided however that such claim within a patent application has not been revoked, cancelled, withdrawn, held invalid or abandoned or been pending for more than [**] years from the date of its first priority filing anywhere in the world;
"Value Added Tax"	shall have the meaning given to it in Clause 13.6; and
"Warranties"	means the warranties given by PTC to the Trust as set out in Clause 18.2.

1.2 References in this Agreement to any statutory provisions shall be construed as references to those provisions as respectively amended consolidated or re enacted (whether before or after the Commencement Date) from time to time and shall include any provisions of which they are consolidations or re-enactments (whether with or without amendment).

1.3 Reference to any statute, statutory instrument, regulation, by law or other requirement of English law and to any English legal term for any actions,

remedy, method of judicial proceeding, legal document, legal status, court, official or any legal concept or doctrine shall, in respect of any jurisdiction other than England, be deemed to include that which most nearly approximates in that jurisdiction to the relevant English term.

1.4 The Schedules and Recitals form part of this Agreement and any reference to this Agreement shall include the Schedules and Recitals.

1.5 In this Agreement:

(a) the masculine gender shall include the feminine and neuter and the singular number shall include the plural and vice versa;

(b) references to persons shall include bodies corporate, unincorporated associations, partnerships and individuals;

(c) except where the contrary is stated, any reference in this Agreement to a Clause or Schedule is to a Clause of or Schedule to this Agreement, and any reference within a Clause or Schedule to a sub Clause, paragraph or other sub-division is a reference to such sub Clause, paragraph or other sub-division so numbered or lettered in that Clause or Schedule.

1.6 The headings in this Agreement are inserted for convenience only and shall not affect the construction of the provision to which they relate.

1.7 References to the winding-up of a person include the amalgamation, reconstruction, reorganisation, administration, dissolution, liquidation, bankruptcy, merger or consolidation of such person and an equivalent or analogous procedure under the law of any jurisdiction in which that person is incorporated, domiciled or resident or carries on business or has assets.

1.8 Any reference to books, records or other information includes books, records or other information in any format or medium including paper, electronically stored data, video or audio recordings and microfilm.

1.9 Any phrase introduced by the terms "including", "include", "in particular" or any similar expression shall be construed as illustrative and shall not limit the sense of the words preceding those terms

1.10 Where reference is made in this Agreement to the prior written consent of the Trust being required in respect of any matter, the Company shall give not less than [**] Business Days notice to the Trust of the matter for which such consent is required.

2. **AWARD**

2.1 The Award Amount will be payable in Tranches and instalments as set out in Schedule 3.

2.2 The Trust shall pay the first instalment of the first Tranche of the Award Amount to PTC within [**] Business Days of the later of:

(a) the Commencement Date; and

(b) the date of written confirmation from the Trust to PTC of acceptance of PTC's then current Treasury Policy.

PTC undertakes to commence the Programme within [**] months of receipt of the first instalment of the first Tranche of the Award Amount. For the avoidance of doubt, the Trust shall not pay any part of the Award Amount to PTC unless and until PTC's then current Treasury Policy has been accepted by the Trust in writing and PTC is in compliance with such Treasury Policy.

2.3 When PTC considers that any Milestone has been achieved by the relevant Milestone Date:

(a) PTC shall as soon as reasonably practicable provide the Trust with a detailed report (the "**Milestone Report**") setting out how the Milestone was achieved and requesting payment of the next Tranche of the Award Amount; and

(b) The Trust shall confirm to PTC in writing, within [**] Business Days of receipt by the Trust of the Milestone Report either that:

(i) the Milestone has been achieved by the Milestone Date to the Trust's reasonable satisfaction, in which case the Trust shall make payment of the next Tranche of the Award Amount within [**] Business Days of the date of the later of:

(A) receipt by PTC of the Trust's confirmation pursuant to this Clause 2.5(b)(i); or

(B) where PTC has amended its Treasury Policy, on the date of written acceptance from the Trust to PTC of the amended Treasury Policy; or

(ii) the Milestone has not been achieved to the Trust's reasonable satisfaction by the relevant Milestone Date and that the payment shall not take place, in which case the Trust shall provide PTC with reasonable details of the grounds on which it has reached this decision.

2.4 The Trust may, at its sole discretion, grant PTC a reasonable period of time ("**Milestone Extension**"), in order to address the reasons why the Trust has judged that a particular Milestone has not been met. Upon the expiry of a Milestone Extension, the Trust shall, at its sole discretion, decide whether or not to permit full or partial payment of the relevant Tranche of funding to PTC.

2.5 PTC shall complete and submit a detailed report on the work done and outcomes of the Programme ("**End of Award Report**") in the prescribed form to the Trust, such report to be presented to the Trust within [**] days after the completion of the Programme (or such other date as may be agreed with the Trust). The Trust will evaluate the End of Award Report and will notify PTC within [**] Business Days of receipt whether the report is acceptable to the Trust. If the End of Award Report is not acceptable to the Trust, it shall notify PTC of its reasons at the same time, which may include that the report is incomplete or insufficiently detailed and the Trust shall have the right to withhold further funding until the Trust receives an End of Award Report which the Trust deems to be acceptable.

2.6 The Trust will only be obliged to pay any Tranche to PTC if, at the time of request for payment from PTC or the due date for payment of the Tranche:

(a) none of the events described in Clauses 20.2, 20.3, 20.4(a), or 20.5 have occurred or would result from the proposed payment;

(b) PTC is not in breach of any of any of the Conditions;

(c) the Warranties are true and correct in all respects, subject to the matters set out in the relevant Disclosure Letter;

(d) PTC's then current Treasury Policy includes provisions ensuring maintenance of the Project funds (including the Loan) in banks with at least the minimum credit rating (e.g. Standard & Poor's) required by the Trust from time to time and such Treasury Policy has been accepted in writing by the Trust;

(e) PTC is in compliance with the most recent Treasury Policy accepted in writing by the Trust;

(f) the Trust has received the relevant Disclosure Letter and the contents of such Disclosure Letter are reasonably acceptable to the Trust; and/or

(g) if so required by the Trust, the Site Visit Group has conducted a review of PTC's facilities in accordance with Clause 6 and such visit has been completed to the reasonable satisfaction of the Trust.

2.7 If any Milestones have not been achieved by [**] months from the Commencement Date, unless agreed otherwise in writing by the Trust, the Trust shall have no obligation to pay any Tranche (or part thereof) which has not been paid prior to that date.

2.8 All payments made by the Trust to PTC under this Agreement shall be made in United States dollars ($ US). PTC shall ensure that it holds a bank account in the currency in which the Award Amount shall be advanced. Payment shall be made by electronic wire transfer of immediately available funds directly to PTC's account designated below or to such other account as PTC may specify by written notice.

Bank Account for PTC:

Account Name: PTC Therapeutics, Inc.

Account No.: [**]

ABA No.: 031201467

Swift No.: PNBPUS33

Bank: Wachovia Bank NA

Branch address: MAC N 2684-020, 120 Mountain View Blvd., Suite 200, Basking Ridge, NJ 07920, USA.

2.9 The contact details of the PTC TPC are set out below. During the Programme Term, PTC shall promptly notify the Trust TPC in writing of any changes to the identity and/or contact details of the PTC TPC.

PTC TPC:

Name:	William Baird, III
Position:	Chief Financial Officer
Address:	100 Corporate Court, S. Plainfield, NJ 07080 USA
Phone number:	1-908-912-9159
Email:	wbaird@ptcbio.com

2.10 The contact details of the Trust TPC are set out below. During the Programme Term, The Trust shall promptly notify the PTC TPC in writing of any changes to the identity and/or contact details of the Trust TPC.

Trust TPC:

Name:	[**]
Position:	[**]
Address:	[**]
Phone number:	[**]
Email:	[**]

2.11 During the Programme Term, in the event that PTC makes any amendments to the Treasury Policy most recently accepted in writing by the Trust, PTC shall prior to such changes taking effect:

(a) notify the Trust TPC in writing of the amendments to the Treasury Policy; and

(b) provide a copy (in English) of the amended Treasury Policy to the Trust TPC for acceptance by the Trust.

The Trust shall promptly notify PTC in writing whether such amended Treasury Policy has been accepted by the Trust.

2.12 In the event that the credit rating of PTC's bank falls to a credit rating below the Trust's minimum required credit rating, the Trust shall not be under any obligation to pay any part of the Award Amount to PTC unless and until PTC operates a bank account with a bank with at least the minimum credit rating required by the Trust from time to time. For the avoidance of doubt, during the Programme Term, the Trust may require PTC to open and operate a bank account with an alternative bank where PTC's original bank's credit rating falls below the minimum credit rating required by the Trust from time to time.

2.13 In connection with each request for payment of a Tranche or instalment thereof from the Trust, PTC shall either:

(a) confirm to the Trust TPC in writing that there have been no amendments to PTC's Treasury Policy most recently accepted in writing by the Trust; or

(b) notify the Trust TPC that:

(i) there have been amendments to the Treasury Policy most recently accepted in writing by the Trust;

(ii) the date such amendments took effect; and

(iii) provide a copy of the amended Treasury Policy (in English) to the Trust TPC for acceptance by the Trust.

2.14 Each of the Trust and PTC shall (and PTC shall procure that relevant members of the PTC Group shall) pay any and all taxes levied in respect of all payments it receives or makes under this Agreement. Any withholding or other taxes that is required by law to be withheld or paid, with respect to any payments to it under this Agreement, shall be deducted from such payments and paid contemporaneously with the remittance, together with evidence of such withholding or payment. Such withholding and payment shall fully discharge the party making the payment and no further payment shall be required by the payor to the payee. The party withholding or making such payment shall furnish the other party with appropriate documents to secure application of the most favourable rate of withholding tax under applicable law.

2.15 PTC undertakes to use all funding received from the Trust pursuant to this Agreement solely for the purposes of the Programme as described in the Application. PTC shall obtain the Trust's prior written consent to any other use of any funding received from the Trust pursuant to this Agreement.

2.16 Subject to Clause 2.17 below, PTC undertakes that it shall not (and PTC shall procure that other members of the PTC Group shall not) seek to apply for or accept without the Trust's prior written consent (such consent not to be unreasonably withheld) any other funding or support (whether in kind or otherwise) for the programme of research agreed for the Programme, whether commercial or non-commercial, during the period of the Programme.

2.17 PTC hereby grants to the Trust a first option to consider any further requirements of PTC for the funding of the programme of research agreed for the Programme or any further development of the results of the Programme for a period of [**] years following the end of the Programme Term. Any such further funding requirements shall be notified to the Trust by PTC (as the case may be), and the Trust shall within [**] days of such notification indicate to PTC whether the Trust wishes to so further fund (in whole or in part), subject to the proper Trust funding application and review process being carried out. If the Trust does not so elect, then the option shall lapse. For clarity, this Clause 2.17 is not intended to restrict PTC from engaging in general capital raising activities provided that such funds are not specifically earmarked for the Programme.

3. **THE PROGRAMME**

3.1 PTC undertakes to use its reasonable endeavours to achieve each Milestone on or before each relevant Milestone Date.

3.2 PTC undertakes to diligently perform the research and the Programme management of the Programme, as set out in the Application and as determined by the RSG and the IPMG from time to time.

3.3 Subject to existing confidentiality obligations and legal restrictions, including contractual restrictions, PTC shall inform the RSG in writing of any on-going research being carried on by PTC or any on-going research that, to the knowledge of PTC, is being carried on by any other member of the PTC Group in the Field. PTC undertakes, throughout the duration of this Agreement, to use its reasonable endeavours to co-operate with and to adopt a synergistic and collaborative approach to work with Third Parties working on similar research and development programmes to the Programme.

4. **PROGRAMME MANAGEMENT AND PROGRAMME AUDIT**

4.1 PTC shall appoint a Programme manager from its Staff who shall be responsible on a day-to-day basis for co-ordinating the internal and external components of the Programme.

4.2 PTC warrants that it has or that it shall have in place contracts with its Staff such that any Programme Intellectual Property shall be owned by and assigned to PTC and that each member of the Staff is obliged to waive (to the extent that such rights exist under applicable law and are waivable) all moral rights and rights of a like nature in the Programme Intellectual Property. The Trust may upon reasonable notice require PTC to produce all and any Staff contracts for inspection by the Trust except as may be limited by applicable laws.

4.3 PTC shall cause to be kept full, detailed and accurate records of all of its activities and results obtained in connection with the Programme. In this respect, PTC shall and shall procure that the Staff shall at all times:

(a) observe professional standards; and

(b) maintain a policy which requires its Staff or others acting on its behalf to record and maintain all data and information developed during the Programme in such a manner as to enable the Parties to use such records to establish the earliest date of invention and/or diligence to reduction to practice (an "**Invention Policy**"). Such Invention Policy shall, among other things, provide that such individuals are to keep record all research, development and other work carried out in respect of the Programme and the results of such research, development and other work, in standard laboratory notebooks (or electronic equivalents that meet the requirements of applicable law) that are dated and corroborated by non-inventors on a regular, contemporaneous basis.

4.4 PTC shall maintain, or cause to be maintained, books and records of its activities under the Programme and its expenditure of the Trust Award (the "**Programme Books and Records**") in sufficient detail and in good scientific

manner appropriate for audit, patent and regulatory purposes, which shall be complete and accurate and shall fully and properly reflect all work done and results achieved in the performance of its respective activities under the Programme, and which shall be retained by PTC for at least [**] years after the creation of the record, or for such longer time period prescribed by PTC's document retention policies or as may be required by applicable law. The Trust shall have the right, during normal business hours and upon reasonable notice, to inspect and copy any Programme Books and Records if required for audit, patent or regulatory purposes provided that the Trust shall not be entitled to exercise this right more than [**] in any calendar year, shall only use such information for the purposes of exercising its rights or complying with its obligations under this Agreement, and shall treat such Programme Books and Records and any copies thereof as Confidential Information.

4.5 PTC shall procure that the control of expenditure to be funded under the Award is governed by the normal accounting standards and procedures applicable to members of the PTC Group and such expenditure shall be covered by the formal audit arrangements that exist within the PTC Group.

Following the annual audit of PTC by its external auditors, PTC shall provide the Trust with the audited financials which shall indicate whether the external auditors have signed their opinion on the annual accounts of PTC without qualification. PTC shall further confirm in writing to the Trust that the management letter from the auditors raises no matters that did or could significantly affect the administration of grants awarded by the Trust. If the auditors have raised any such matters in their management letter, PTC shall, on request from the Trust, provide the Trust with relevant extracts from the letter.

4.6 During the Programme Term and for a period of [**] years afterwards, the Trust shall have the right, at its discretion and expense, to audit (either directly or via a Third Party engaged by it):

(a) any expenditure of the Award Amount including, without limitation, any expenditure by PTC, any other member of the PTC Group, Co-applicant, collaborators and/or subcontractors;

(b) the systems used by PTC to administer Trust grants generally; and

(c) any equipment acquired under the Award Amount,

provided that the Trust shall not be entitled to exercise this audit right more than [**] in any calendar year, and shall only use such information for the purposes of exercising its rights or complying with its obligations under this Agreement, and shall treat the any documents reviewed or information received as a result of such audit as Confidential Information.

4.7 In furtherance of the Trust's audit right pursuant to Clause 4.6, PTC shall (and shall procure that its Affiliates shall) provide access at any time during business hours for auditors and other personnel from or appointed by the Trust to accounting and other financial and corporate records relating to this Agreement, the Award, the Programme Books and Records, and the activities funded by the Trust (at the Trust's expense), if requested at any time on reasonable advance notice. Where any of the Award Amount has been paid by PTC to any collaborators and/or sub-contractors, PTC shall use its best

efforts to procure that the right of access for audit purposes extends to the accounts and records of any such collaborator and subcontractor.

4.8 PTC shall maintain a separate accounting cost code specific to the Award, and all costs and income properly relating to the Award shall be accounted for through that cost code. PTC shall ensure that appropriate records are kept to support the entries made on the cost code.

4.9 PTC shall be responsible for the management, monitoring and control of all research work undertaken by it. This shall include, as appropriate, the requirements of all applicable laws and regulatory authorities, including but not limited to those governing the use of radioactive isotopes, diagnostic tools, animals, pathogenic organisms genetically modified organisms, toxic and hazardous substances, research on human subjects and human embryos, and include appropriate ethical approvals and consents, including for example but not limited to, such approvals and consents for obtaining tissues and other human samples. For any clinical trial carried out pursuant to the Programme, PTC shall on the Trust's written request supply details of such clinical trial for publication on the Trust's clinical trials register and any applicable national clinical trials register.

4.10 Any research under the Programme that involves animals that is undertaken by any member of the PTC Group, collaborators, subcontractors or service providers (whether in the UK, United States or any other country) shall comply with both the Trust's policy on the use of animals in research and the principles set out in the document "Responsibility in the use of animals in bioscience research: Expectations of the major research council and charitable funding bodies" (http://www.wellcome.ac.uk/About-us/Policy/Policy-and-position-statements/WTD040129.htm). If procedures taking place in the UK and regulated under the UK Animals (Scientific Procedures) Act 1986 will be used, the research shall comply with such Act, be approved by the local ethical review process and be conducted with due consideration for the 3Rs (replacement, reduction and refinement of the use of animals in research). If procedures taking place in the US and regulated under the US Animal Welfare Act of 1966, as amended, the research shall comply with such Act.

4.11 Any research under the Programme that is undertaken by any member of the PTC Group, collaborators, subcontractors or service providers (whether in the UK, the United States or any other country) shall:

(a) comply with the World Medical Association's "Declaration of Helsinki - Ethical Principles for Medical Research Involving Human Subjects 2008" as amended from time to time;

(b) be subject to appropriate ethical review procedures in accordance with applicable law;

(c) be covered by an appropriate insurance policy or by appropriate indemnity arrangements, to the extent commensurate with such research;

(d) comply with all applicable local legislation; and

(e) be approved by the local ethical review process.

5. THE RESEARCH STEERING GROUP

5.1 As soon as practicable following the Commencement Date, the Trust and PTC shall establish a Research Steering Group ("**RSG**") to oversee the Programme, which shall:

(a) monitor the performance and technical content of the Programme against the description outlined in the Application;

(b) assess the ongoing results of the Programme and what has been learnt and agree future research;

(c) critically assess the results of the Programme;

(d) identify and address any weaknesses or delays in the Programme;

(e) co-ordinate internal and outsourced components of the Programme, including agreeing on whether to pursue any collaborations or sub-contracts not specifically identified in the Application;

(f) modify or authorise modifications to the implementation of the Programme (including the implementation of the Programme objectives) as necessary from time to time;

(g) operate as the key forum through which the Trust shall be informed as to progress of the Programme and through which the Trust shall liaise with PTC concerning the conduct of the Programme, including preparing an annual written report for the Trust on progress;

(h) advise the Trust when and whether each of the research phases, Milestones or targets of the Programme have been achieved; and

(i) review all proposed public disclosures relating to the Programme, including proposed presentations, posters and papers (ensuring that the contribution of the Trust is acknowledged in all such proposed publications and that the Trust's Award number is quoted) and advise the Trust as to the RSG's reasonable recommendations in respect of such proposed publications;

provided that the RSG shall have no right to amend or vary the terms of this Agreement or to alter the fundamental scope or objectives of the Programme which power is reserved to the Parties.

5.2 The RSG shall be established and run by the Parties as follows:

(a) The RSG shall comprise the following persons ("**Members**"):

(i) [**] representatives of PTC, one of whom shall be the Principal Investigator;

(ii) at least one independent industry adviser with experience which is relevant to the Programme; and

(iii) [**] representatives or nominees of the Trust's Technology Transfer Division (at the Trust's option).

(b) The Trust shall have the option to appoint up to [**] Members and up to [**] observers to the RSG, to remove any Member or observer appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such Member or observer. In the event that the Trust does not appoint any Member or observer, the Trust shall have the right to receive all papers that a Member or observer would be entitled to receive.

(c) PTC shall have the option to appoint up to [**] Members and up to [**] observers to the RSG, to remove any Member or observer appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such Member or observer; provided, that the Principal Investigator shall always be one of PTC's Members.

(d) PTC and the Trust shall jointly agree the identity of the Members of the RSG who are independent industry adviser(s). The costs and expenses of the independent industry adviser(s) shall be met out of the Award Amount.

(e) The Principal Investigator shall be the chairperson of the RSG ("**RSG Chair**") and shall be responsible for organising meetings of the RSG, including preparing papers prior to meetings and ensuring that minutes of meetings are produced promptly after each meeting. All papers and minutes shall be circulated to each Member in a timely manner. Except in exceptional circumstances (when the Principal Investigator may nominate another person as his alternate), the Principal Investigator shall attend all RSG meetings.

(f) The quorum for RSG meetings shall be [**] Members including [**]. Decisions of the RSG shall be made by majority agreement with each Party entitled to cast one (1) vote regardless of the number of Members present. If the RSG is unable to reach agreement on a decision, the decision shall be escalated to the Director of Technology Transfer at the Trust and the head of Discovery Research at PTC for resolution. For the avoidance of doubt, any observers appointed by the Parties shall not be Members of the RSG and shall not have a right to participate in its decision-making process unless otherwise agreed in writing by PTC and the Trust.

5.3 Meetings of the RSG shall be convened by the Principal Investigator as required but at least [**] months (or less frequently with the consent of the Trust) for the duration of the Programme Term, on not less than [**] Business Days' written notice (to be accompanied by an agenda for the meeting). Following the end of the Programme Term, the RSG shall meet within [**] Business Days to discuss and report on the outcomes of the Programme, and shall thereafter be dissolved.

5.4 Any or all Members may, with the prior consent of the RSG Chair, attend a meeting of the RSG by telephone or other electronic means rather than in person, provided that all Members attending the meeting can hear and be heard for all parts of the meeting. For the avoidance of doubt, RSG Members

attending a meeting by telephone or other electronic means shall have the same voting rights as an RSG Member present in person.

5.5 A representative from any key outsourcing suppliers, collaborators or subcontractors involved in the Programme (if any) shall be invited to RSG meetings as an observer. The RSG shall also have power to invite persons whose special skills or influence might advance the Programme to attend and address meetings of the RSG. Such persons shall not be Members of the RSG and shall not have a right to participate in its decision-making process. The RSG Chair shall ensure that any such invitees sign confidentiality agreements in a form acceptable to all parties.

5.6 PTC shall upon request make available to the Trust and/or the RSG copies of all records generated in connection with the Programme, including for the avoidance of doubt, records generated by its Staff under Clause 4.3 and by any Third Party collaborators to the Programme appointed under Clause 7.

5.7 During the Programme Term, PTC shall procure that the Principal Investigator monitors the work carried out under the Programme for material that may be the subject of Programme Inventions and shall promptly notify the RSG of any such Programme Invention. Without prejudice thereto, during the Programme Term, PTC shall make reports on work being carried out under the Programme to the RSG [**], or from time to time as the RSG may reasonably request, such reports to include the progress of the Programme as well the matters described in Clause 11.9.

6. **SITE VISIT GROUP**

6.1 The Trust may appoint a Site Visit Group, made up of a small team of independent experts and observers from the Trust's Technology Transfer Division. The Site Visit Group shall have reasonable access for the duration of the Programme during normal working hours and at mutually agreed times to visit all the premises where the Programme is being conducted to consult informally with PTC's employees, researchers, consultants or contractors working on the Programme to evaluate progress, performance and key issues and to report back to the Trust and the RSG on their findings.

6.2 The Site Visit Group may recommend that the Trust terminates the Programme due to a serious failure in the progress, management or conduct of the Programme (including but not limited to a finding that the Programme will be unable to achieve the next Milestone within a reasonable time period after the relevant Milestone Date), or due to a major external scientific, technical or commercial barrier which means that the Programme is unlikely to succeed in its objectives. If the Site Visit Group makes such a recommendation pursuant to this Clause 6.2, it must provide written notice of its recommendation and the rationale therefor to the Parties.

6.3 If PTC is unable to remedy a serious failure or external barrier identified by the Site Visit Group pursuant to Clause 6.2 within [**] Business Days, or such longer time period as the Trust may, in its sole discretion, allow, the Trust may terminate this agreement pursuant to Clause 20.3(b).

7. **PROGRAMME COLLABORATORS AND SUBCONTRACTORS**

7.1 If PTC wishes to use a Third Party collaborator or sub-contractor to conduct any part of the Programme, it shall seek the consent of the RSG unless such sub-contractor or collaborator is specified in the Application. PTC shall provide a copy of the agreed form of any sub-contract or collaboration agreement to the Trust for review by the Trust prior to signature by the parties thereto. Unless otherwise agreed in writing between the Parties, PTC shall ensure in all cases that any collaborations or sub-contracts shall be on the following terms:

(a) that the Third Party shall not have any rights to any results emerging from such work and all such results shall as between the Parties and the Third Party be deemed to be Programme Intellectual Property and owned in accordance with the provisions of this Agreement; provided, however, that if applicable law prevents assignment of ownership, PTC shall use its best efforts to secure appropriate license or option rights to such intellectual property;

(b) that the Third Party shall be under obligations of confidence concerning such results on terms equivalent to those set out under this Agreement;

(c) that the Third Party shall keep detailed records including scientific notebooks of all of its activities and upon request shall make available copies to the Trust, except where prohibited by applicable law;

(d) that the Third Party will upon reasonable request make available its premises where the Programme is being conducted, and its employees and/or consultants for discussion with the Site Visit Group as referred to in Clause 6, except where prohibited by applicable law; and

(e) that the provisions of such sub-contract or collaboration agreement shall be consistent with the nature of the Award and the payment of the Award Amount in Tranches, the Trust's rights pursuant to Clauses 12 and 13 and the termination provisions of this Agreement, and shall terminate if this Agreement terminates.

8. **INTELLECTUAL PROPERTY — OWNERSHIP AND PROTECTION**

8.1 In the event that any Programme Intellectual Property arises, it shall be the property of PTC. Any Programme Patents arising from such Programme Intellectual Property shall be applied for in the name of PTC. PTC shall have the first option to take responsibility for seeking and maintaining protection for Programme Intellectual Property in consultation with the RSG at PTC's sole cost, including the filing, conduct, prosecution and maintenance of all patents arising in respect of Programme Inventions.

8.2 If PTC chooses not to pursue filing, prosecution or maintenance of any Programme Patents in any country, it shall immediately notify the Trust of this fact in writing. The Trust shall be entitled, but not obliged, at its own cost, to pursue or maintain such Programme Patents in the relevant country or countries in PTC's name and PTC shall provide such assistance to the Trust

at the Trust's sole cost as may reasonably be required by the Trust in order to do so.

8.3 Without prejudice to the terms of this Clause 8.3, PTC shall (and shall procure that the Principal Investigator shall) execute such further documents, take such action and do such things as may be reasonably requested by the Trust at the Trust's cost to secure the right of the Trust to protect, maintain, manage, defend, enforce and exploit the Programme Intellectual Property referred to in this Clause 8 and Clauses 9, 10, 11 and 12 below.

8.4 PTC shall make the Background Intellectual Property available for use in the Programme and for the protection, development and exploitation of the Programme Intellectual Property. PTC shall, unless otherwise agreed, retain responsibility for seeking and maintaining protection for the Background Intellectual Property at its own cost. If PTC chooses not to pursue filing, prosecution, maintenance, defence or enforcement of any patent rights that are Background Intellectual Property in any country, they shall give the Trust at least [**] months' notice of this fact in writing. During the [**]-month notice period, PTC shall continue to seek and maintain such patent rights. The Trust shall be entitled, but not obliged, at its own cost, to assume responsibility (on behalf of PTC) for filing, prosecuting, maintaining, defending or enforcing such patent rights in the relevant country or countries in PTC's name and PTC shall provide such assistance to the Trust at the Trust's cost as may reasonably be required by the Trust in order to do so.

8.5 The Trust acknowledges that portions of the PTC Previous Research were funded by the US Government. Notwithstanding anything in this Agreement to the contrary, the rights of the Trust with respect to Background Intellectual Property shall be subject to the rights of the US Government in such Background Intellectual Property.

9. **INTELLECTUAL PROPERTY MANAGEMENT GROUP**

9.1 PTC and the Trust shall establish an Intellectual Property Management Group ("**IPMG**"), which shall:

(a) approve all public disclosures relating to the Programme, including presentations, posters and papers (provided that the contribution of the Trust is acknowledged in all such publications and quoting the Award number);

(b) identify new inventions arising out of the Programme and make recommendations for IP strategy, including patent filing and prosecution strategy and assessment of freedom to operate issues; and

(c) approve the Exploiting Party's Development and Exploitation strategy in relation to the Programme Intellectual Property.

9.2 The IPMG shall be established and run by the Parties as set out below:

9.3 The IPMG shall be comprised of the following persons ("**IPMG Members**"):

(a) not more than [**] representatives of PTC;

(b) not more than [**] representatives of the Trust's Technology Transfer Division or their nominees.

9.4 The Trust shall have the option to appoint [**] IPMG Members, to remove any IPMG Member appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such IPMG Member. In the event that the Trust does not appoint such IPMG Members, the Trust shall have the right to receive all papers that an IPMG Member would be entitled to receive.

9.5 PTC shall have the option to appoint [**] IPMG Members, to remove any IPMG Member appointed by it and to appoint any person to fill a vacancy arising from the removal or retirement of such IPMG Member. If PTC does not appoint such IPMG Members, PTC shall have the right to receive all papers that an IPMG Member would be entitled to receive.

9.6 The IPMG Members shall select a chair ("**IPMG Chair**") and the IPMG Chair shall be responsible for organising meetings of the IPMG, including preparing papers prior to meetings and ensuring minutes of meetings are produced. All papers and minutes shall be circulated to each IPMG Member in a timely manner.

9.7 The quorum for IPMG meetings shall be [**] IPMG Members, provided that at least [**]. Decisions of the IPMG shall be made by majority agreement with each Party entitled to case one (1) vote regardless of the number of IPMG Members present. If the IPMG is unable to reach agreement on a decision, the decision shall be escalated to Director of Technology Transfer at the Trust and the General Counsel of PTC for resolution. If the IPMG Chair is unable to attend an IPMG meeting, PTC and the Trust shall, in good time before the meeting, nominate an alternative IPMG member to act as Chair.

9.8 Meetings of the IPMG shall be convened by the IPMG Chair at least [**] per year and otherwise on an "as needed" basis, either in person at the premises of PTC or by 'virtual private network' videoconference if necessary. Any or all IPMG Members may, with the prior consent of the IPMG Chair, attend a meeting of the IPMG by telephone or other electronic means rather than in person, provided that all IPMG Members attending the meeting can hear and be heard for all parts of the meeting. For the avoidance of doubt, IPMG Members attending a meeting by telephone or other electronic means shall have the same voting rights as an IPMG Member present in person.

10. **INFRINGEMENT**

10.1 Each Party shall immediately give notice to the other Party if any member of the Trust, the PTC Group or their Staff become aware of:

(a) any infringement of the Background Intellectual Property or Programme Intellectual Property; or

(b) any claim by a Third Party that an action carried out under the Programme infringes the Intellectual Property or other rights of any Third Party.

10.2 In respect of any Background Intellectual Property or Programme Intellectual Property, where any infringement or suspected infringement arises, or a claim

by a Third Party alleging infringement of that Third Party's Intellectual Property or other rights arises, then:

(a) As soon as possible after receiving the notice required by Clause 10.1, the Parties will convene a meeting of the IPMG at which the IPMG shall discuss in good faith all available evidence with respect to the matters underlying the notice, and the appropriate manner of addressing such matters, including preventing or stopping infringing activities (for example, by seeking a preliminary injunction), preserving the Parties' rights to past and future damages (for example, by sending a cease and desist letter) defending against declaratory judgment actions with respect thereto, or taking any other actions, or no actions, as the Parties shall determine. The IPMG shall take into account each Party's interest in formulating the response, if any, to infringement or threatened infringement of such Background Intellectual Property or Programme Intellectual Property, including the relative merits of patent litigation versus the nature, scope and potential economic consequences of the Infringement.

(b) Unless otherwise determined by the IPMG as part of its consideration of an overall patent strategy, or pursuant to its evaluation of a notice pursuant to Clause 10.2(a), if a member of the PTC Group (or any licensee of a member of the PTC Group) is exploiting the relevant Programme

Intellectual Property but PTC notifies the Trust that it does not intend to take such action, the Trust, at its discretion and cost may take such action as it shall consider to be necessary or appropriate to bring or defend an action on behalf of the relevant member of the PTC Group or licensee thereof and PTC shall (and shall procure that relevant members of the PTC Group shall) provide all reasonable assistance to Trust as the Trust may request (at the Trust's cost); and

(c) Unless otherwise determined by the IPMG as part of its consideration of an overall patent strategy, or pursuant to its evaluation of a notice pursuant to Clause 10.2(a), if the Trust (or any licensee of the Trust) is exploiting the relevant Programme Intellectual Property following exercise of its rights pursuant to Clause 12, the Trust may take such action as it shall consider to be necessary or appropriate at its discretion and expense to bring or defend an action on behalf of the relevant member of the PTC Group. PTC shall (and PTC shall procure that the relevant member of the PTC Group shall) give the Trust all reasonable assistance as the Trust may request (at the Trust's cost) in relation to such action, including granting the Trust the right to bring such action in PTC's name.

10.3 In the event that any enforcement or defence action whether by a member of the PTC Group and/or the Trust results in the recovery of legal costs and/or an award of damages, such sums shall be distributed in accordance with the following the following order of priority: (a) first, to reimburse each Party for all litigation costs in connection with such proceeding paid by that Party and not otherwise recovered (on a pro rata basis based on each Party's respective litigation costs, to the extent the recovery was less than all such litigation costs); and (b) second, [**] percent ([**]%) to the enforcing Party and [**] percent ([**]%) to the non-enforcing Party.

10.4 Notwithstanding the above, except as may be agreed otherwise by the Parties following good-faith discussions, no Party shall enforce their rights under any Programme Intellectual Property for infringement or potential infringement by:

(a) any organisation operating on a Not for Profit Basis or any charitable organisation which is conducting non-commercially sponsored research; and/or

(b) any person carrying out non-commercially sponsored research on behalf of any organisation operating on a Not for Profit basis or any charitable organisation.

11. EXPLOITATION

11.1 To the extent that PTC finds it necessary or useful to acquire or license rights from Third Parties in order to use the Programme Intellectual Property for Development and Exploitation in the Field, PTC shall use commercially reasonable efforts to ensure that it has, or has the right to acquire, the ability to grant such rights to the Trust if the Trust becomes the Exploiting Party. For the avoidance of doubt, nothing in this Clause 11.1 shall be construed as a warranty or representation that any Products will be launched or that the use of Programme Intellectual Property for Development and Exploitation will not infringe any Third Party rights.

11.2 Each Party agrees that it shall promptly, (and in the case of PTC, will procure that the relevant member of the PTC Group shall promptly), inform and deliver written details to the other Parties of, any safety concerns or issues raised by any Competent Authority that relate to the Products.

11.3 Prior to any member of the PTC Group (whether itself or through any other member of the PTC Group, or by granting a license or in collaboration with any Third Party) (the "**Exploiting Party**"), commencing the Development and/or Exploitation of any Programme Intellectual Property and/or Products both inside and outside the Field, PTC or the relevant the member of the PTC Group shall obtain the prior written consent of the Trust to such Development and Exploitation by sending written notice to the Trust and the following information:

(a) reasonable details of the relevant Programme Intellectual Property, the Products and the activity proposed;

(b) details of whether the proposed Exploitation will be on a For-Profit and/or Not-For-Profit Basis; and

(c) if applicable, amounts of any milestones payments and royalties that would be payable to the Trust pursuant to Schedule 6 and any other applicable terms.

11.4 Where Exploitation is to be on a For-Profit Basis, the grant of the Trust's consent pursuant to Clause 11.6 shall be conditional on the payments to the Trust of amounts calculated pursuant to Schedule 6, and the Trust and PTC agreeing an appropriate share of any revenue payable to the Trust pursuant to Schedule 6.

11.5 In the event that the relevant member of the PTC Group Develops and/or Exploits the Programme Intellectual Property and/or Products in the Field on a Not for Profit Basis, no amounts shall be payable to the Trust in respect of such Development and/or Exploitation.

11.6 The Trust shall notify PTC in writing within [**] days of the receipt of the notice from PTC as to whether it consents (such consent not to be unreasonably withheld) to the Development and Exploitation of the Programme Intellectual Property and/or Products inside or outside the Field. Following receipt of such consent, PTC shall be free to Develop and Exploit the relevant Programme Intellectual Property in accordance with the consent given by the Trust without further consent or approval from the Trust. If, in respect of any Programme Intellectual Property, the Trust does not give its consent, the Parties shall meet to discuss the Trust's concerns and if they are unable to resolve those concerns the matter shall be referred to the dispute resolution procedure set out in Clause 19. All agreements entered into by PTC relating to the Programme Intellectual Property shall be consistent with the terms of this Agreement.

11.7 If PTC (either by itself, through any other member of the PTC Group, or through a Distributor) decides, at its own discretion not to Develop and Exploit any discrete part of the Programme Intellectual Property, PTC shall take reasonable steps to identify potential Licensees of the Programme Intellectual Property to Develop and Exploit Products. If neither PTC nor any other member of the PTC Group (either by itself or through a Distributor or Licencee) takes reasonable steps to Develop and Exploit the Programme Intellectual Property, that Trust shall have the rights set out in Clause 12.

11.8 The Parties acknowledge that the Exploiting Party or any licensee of the Exploiting Party may be liable to pay royalties and make other payments to Third Parties (including members of the PTC Group) in respect of the Development and Exploitation of the Programme Intellectual Property and/or Products. The Exploiting Party agrees that it or the relevant licensee shall be solely responsible, at their own cost, for all such payments to Third Parties and any amounts payable to the other Parties under this Agreement shall not be reduced as a consequence except as explicitly provided in Schedule 6.

11.9 During the Programme Term, PTC shall keep the Trust reasonably informed on all matters relating to the Development and Exploitation of the Programme Intellectual Property and Products by or on behalf of PTC via the [**]RSG reports required pursuant to Clause 5.7. Following the Programme Term, PTC shall provide all matters relating to the Development and Exploitation of the Programme Intellectual Property and Products by or on behalf of PTC [**].

12. TRUST STEP-IN RIGHTS

12.1 Subject to Clause 12.4:

(a) if no member of the PTC Group or sub-licensees of the PTC Group is taking reasonable steps to Develop or Exploit any Programme Intellectual Property or Products for a particular indication for a consecutive period of [**] months or more following completion of the Programme, and, upon receipt of a written notice from the Trust served at the end of, or after such [**] month period requesting that the Programme Intellectual Property is Developed and/or Exploited,

does not for an additional [**] months take any reasonable steps in this regard; or

(b) at any time after the first sale of a Product in a Major Market in a particular indication, no PTC Group member or any sub-licensees of any PTC Group member have taken reasonable steps to Develop and/or Exploit that Product in that particular indication in another Major Market for a consecutive period of [**] months, and, upon receipt of written notice from the Trust after such [**] month period requesting that the relevant Product is Exploited in such other region(s) and in such indication, does not for an additional [**] months take reasonable steps in this regard,

then, following the expiry of the time periods set out above, the Trust shall have the option in its sole discretion by giving written notice to PTC to become the Exploiting Party and to take responsibility for the Development and Exploitation of such Programme Intellectual Property and Products in the relevant indication(s) and region(s), which includes discretion to make any and all decisions (in consultation with the RSG) regarding the negotiation, acceptance and conclusion of terms for any agreement regarding the Development and Exploitation of such unexploited Programme Intellectual Property (including Development and Exploitation by way of licence, sale, materials transfer or other transfer of rights, as well as any transaction which involves placing such unexploited Programme Intellectual Property into a separate corporate vehicle) in such region.

12.2 If the Trust exercises its right to exploit any Programme Intellectual Property under Clause 12.1:

(a) PTC will licence to the Trust or its nominee, the Programme Intellectual Property in such indications or regions as are specified in the notice served by the Trust exercising the option consistent with the applicable sub-clause in Clause 12.1. The terms of such licence to the relevant Programme Intellectual Property shall:

(i) be free of consideration in respect of sales of Product made on a Not-for-Profit Basis,

(ii) include a share of any revenue or other consideration received by the Trust under any license of relevant Product Intellectual Property with respect to all other sales, such share to be based on the respective contributions made by PTC and the Trust in the Development and Exploitation of such Product, and

(iii) be exclusive, subject to any license, option or other third party rights under any agreement entered in to after the Commencement Date consistent with the terms of this Agreement;

(b) PTC will grant to the Trust or its nominee, a non-exclusive licence to relevant Background Intellectual Property solely as required and for the purposes of enabling the Trust to exercise the rights to the relevant Programme Intellectual Property as described in (a) above and solely in the regions specified in the notice served by the Trust

exercising the option; provided, that such non-exclusive license (1) shall be subject to the rights of the US Government as set forth in Clause 8.5 and (2) shall be subject to any license, option or other third party rights under any agreement entered in to after the Commencement Date consistent with the terms of this Agreement. Any such licence grant shall be non-exclusive and free of charge other than for reasonable costs that are incurred in respect of necessary third-party licences; and

(c) provide the Trust with access to any associated data, Documents (including, without limitation, Documents relating to pre-clincial data and clinical trials), pre-clinical data, Materials (only to the extent actually in existence and amenable to transfer in reasonable quantities without further regulatory approval(s), and not to include commercial inventory of Product for which PTC retains rights to Exploit), regulatory approvals, Marketing Approvals, or information as required for the Trust to exploit such rights.

12.3 If the Trust exercises its right to exploit any Programme Intellectual Property under Clause 12.1 above, PTC agrees that it shall pass (or will procure that relevant members of the PTC Group shall pass) to the Trust immediately any or all exploitation opportunities in the relevant indication(s) and region(s) that it becomes aware of from time to time in connection with the Programme Intellectual Property. PTC further undertakes that it shall not (and that it shall procure that no member of the PTC Group shall) engage in any activities (including in relation to the Background Intellectual Property) that could reasonably lead to the loss of an exploitation opportunity in the applicable region and with respect to the applicable indication(s) without the prior written consent of the Trust.

12.4 Notwithstanding anything to the contrary set forth in this Clause 12, in the event that PTC or a member of the PTC Group licenses a Third Party to exploit the Programme Intellectual Property (whether alone or together with other Intellectual Property of any member of the PTC Group) in any indications and in any regions then the Trust shall have no rights under this Clause 12 with respect to such Programme Intellectual Property in such indications and in such regions, where:

(a) under a written agreement with a member of the PTC Group, such licensee is required to use diligent efforts to exploit the licensed Programme Intellectual Property in the relevant indication in the relevant region(s), and such written agreement provides for a reversion to the relevant member of the PTC Group of the Programme Intellectual Property in such indication and in such region(s) if the licensee materially breaches this diligence obligation; or

(b) the Trust has approved such licence in writing.

12.5 The Exploiting Party shall determine the regulatory plans and strategies and clinical trials ("**Key Product Strategy**") for any Products and shall be responsible for filing all regulatory filings with respect to the Products and will be responsible for obtaining and maintaining regulatory approvals in the name of the Exploiting Party. The Exploiting Party shall keep the Non-Exploiting Party informed regarding the Key Product Strategy for each Product and take into account the reasonable recommendations of the Non-Exploiting Party

relating to such Key Product Strategy. Notwithstanding the above, the Parties acknowledge and agree that if the Non-Exploiting Party can reasonably demonstrate that any aspect of the Key Product Strategy proposed by the Exploiting Party will materially prejudice the Exploitation of any Product(s) which have been launched, the Exploiting Party will not proceed with such aspect of the Key Product Strategy.

13. **REVENUE PAYMENTS**

13.1 Unless otherwise agreed between the Parties in writing, all payments due to the Trust shall be made in to the following account:

Account Name:	The Wellcome Trust
Bank name:	[**]
Bank Address:	[**]
Sort Code:	[**]
Account No:	[**]
IBAN:	[**]
BIC:	[**]

13.2 Except as expressly provided herein or otherwise agreed between the Parties in writing, all payments due to PTC under this Clause 13 shall be made in to the following account:

Account Name:	PTC Therapeutics, Inc.
Account No.:	[**]
ABA No.:	031201467
Swift No.:	PNBPUS33
Bank:	Wachovia Bank NA
Branch address:	MAC N 2684-020, 120 Mountain View Blvd., Suite 200, Basking Ridge, NJ 07920, USA.

13.3 Within [**] days of the end of each Quarter, the paying Party shall deliver a statement to other Party setting out all sales of Product made by the paying Party, any member of the paying Party's Group or any Third Party in the relevant Quarter and the amount of revenue and any payment under Clauses 12 and 13 which is due to the receiving Party (**"Quarterly Statement"**). The receiving Party shall deliver to the paying Party an invoice for the amount due as set out in the Quarterly Statement in United States dollars. The revenue amount and any other amount invoiced shall be payable to the receiving Party within [**] days of receipt of the invoice.

13.4 With respect to amounts invoiced in United States dollars, all such amounts shall be expressed in United States dollars and shall be payable in United States dollars. With respect to amounts invoiced in a currency other than United States dollars, all such amounts shall be expressed, for information purposes only, in United States dollars as well as in the currency in which the amount was invoiced and shall be payable in the currency in which the amount is invoiced. The United States dollars equivalent shall be calculated using the paying Party's then current standard exchange rate methodology applied in its external reporting or the conversion of foreign currency sales into United States dollars, in each case as applied consistently throughout the paying Party's organisation.

13.5 If a Party does not receive payment of any sums due to it under this Clause 13 within the time specified, interest shall accrue on such sums at the rate equivalent to US LIBOR 3 months + [**], calculated on a daily basis.

13.6 All payments due under this Clause 13 are expressed to be exclusive of goods, sales, value added or any similar tax (**"Value Added Tax"**) howsoever arising, and the paying Party shall pay the receiving Party, in addition to those payments, all Value Added Tax for which the receiving Party is liable to account to any Competent Authority in relation to any supply made or deemed to be made for Value Added Tax purposes pursuant to this Agreement. The paying Party shall pay any payments due to the receiving Party under this Clause 13.6 at the same time as the relevant payment is due under this Agreement.

13.7 The obligation of the Trust to pay PTC the Award Amount in accordance with Clause 2 and the obligation of PTC and the members of the PTC Group to pay the Trust the revenue and any other payments in accordance with Clauses 12 and 13 shall be material obligations of this Agreement for the purposes of Clause 20.2 (a).

14. **AUDIT OF REVENUE DUE**

14.1 The Exploiting Party shall keep legible, true and accurate records and books of account for [**] years following the end of the calendar year to which they relate and procure that any affiliate of the Exploiting Party which is Exploiting the Programme Intellectual Property and any licensees of the Programme Intellectual Property shall keep legible, true and accurate records and books of account for [**] years following the end of the calendar year to which they relate, which contain all data necessary for the calculation of the revenue payable by it to any other Party (the "**Books**").

14.2 The Non-Exploiting Party shall have the right for a period of [**] years after receiving any report or statement with respect to royalties due and payable to appoint an internationally-recognized independent accounting firm (the "**Auditor**") reasonably acceptable to the Exploiting Party to inspect the Books to verify such reports, statements, records or books of accounts, as applicable. Before beginning its audit, the auditor shall execute an undertaking acceptable to the Party being audited by which the auditor shall keep confidential all information reviewed during such audit. The auditor shall have the right to disclose to both the Party arranging the audit and the Party whose books have been audited, its conclusions regarding any payments owed to such Party.

14.3 The audited Party shall (and shall procure that its Affiliates and any licensees of the Programme Intellectual Property shall) make their records available for inspection by such auditor during regular business hours at such place or places where such records are customarily kept, upon receipt of reasonable advance notice from the Party arranging the audit, solely to verify the accuracy of sales reports, payments records or books of accounts and compliance in other respects with this Agreement. Such inspection right shall not be exercised more than [**] in any calendar year. The Party arranging the audit agrees to hold in strict confidence all information received and all information learned in the course of any audit or inspection, except to the extent necessary for such Party to reveal such information in order to enforce

its rights under this Agreement or if disclosure is required by law, regulation or judicial order.

14.4 The Party arranging for the audit shall pay for such inspections, as well as its own legal expenses associated with enforcing its rights with respect to any payments hereunder, except that in the event there is any upward adjustment in aggregate amounts payable for any year shown by such inspection of more than [**] per cent ([**]%) of the amount paid, in which case the audited Party shall pay for such inspection.

15. PUBLICATIONS

15.1 So as not to jeopardise any Programme Patent filing or exploitation activity being undertaken, PTC shall (and shall procure that any member of the PTC Group or Licencee shall) provide the Trust with copies of any proposed publication or presentation which relates to a Programme Invention or Programme Intellectual Property in advance of the submission of such proposed publication or presentation to a journal, editor or publication. The Trust shall have at least [**] Business Days from and including the date of receipt from PTC of any proposed publication or presentation to object to the same because there is patentable subject matter relating to the Programme Invention that needs protection or because such publication would materially jeopardise any Exploitation activity. The Trust will not seek to withhold consent where such publication or presentation will not prejudice the protection or Exploitation of the Programme Intellectual Property and/or the Products.

15.2 In the event that the Trust objects to any such publication or presentation on the basis that it would disclose patentable information, PTC shall refrain (and shall procure that members of the PTC Group, any licensees, the Principal Investigator and the Staff also refrain), from making such publication or presentation for a period of [**] days from date of receipt of such objection in order for PTC to file the relevant patent application(s) with respect to the patentable subject matter contained in the proposed publication or presentation. Following the expiry of such [**] day period or, if earlier, publication of any patent filed by PTC**,** PTC shall have the right to publish and reproduce any such publication freely with due acknowledgement of the source.

15.3 A copy of the final manuscript of all research publications that relate to the Programme must be made available from PubMed Central (or UK PubMed Central) as soon as possible and in any event no later than [**] months after publication.

16. ANNOUNCEMENTS

16.1 Save for the information described in Clause 16.2 or as required by law or any competent regulatory authority no announcement concerning this Agreement or its subject matter shall be made by the Parties without the prior written approval of both Parties. For clarity, once an item of information concerning this Agreement or its subject matter becomes public in compliance with the terms of this Agreement (for example, by an agreed press release or scientific publication), it may be used in public communications by either Party without the need for consent of the Parties.

16.2 The Trust may publish summary details of the Programme including the name of the Principal Investigator, the name of PTC, the title of the Programme, the Award Amount and the following description of the Programme:

PTC Therapeutics, Inc. (PTC) is targeting the treatment of infections caused by multidrug-resistant Gram-negative bacteria, which represents an area of unmet medical need. PTC has identified a novel structural class of molecules that selectively inhibit bacterial DNA synthesis and have bactericidal activity. These molecules are active predominantly against Gram-negative bacteria, although several analogs in the series also have activity against Gram-positive species, including methicillin-resistant S. aureus (MRSA). PTC compounds are potent against Gram-negative bacteria that are resistant to marketed antibiotics. Representative compounds have good pharmaceutical properties and are efficacious in murine models of systemic E. coli infection. The anti-infective program at PTC is currently in lead optimization and advancing towards identifying a development candidate as a potential first-in-class drug for the treatment of life-threatening infections caused by Gram-negative multidrug-resistant bacteria.

16.3 The Trust's contribution must be acknowledged in all scientific publications concerning the Programme, quoting the Award reference number.

17. CONFIDENTIALITY

17.1 Subject to Clauses 17.2 to 17.7 inclusive below, each Party undertakes that both during and for a period of [**] years after termination of this Agreement, it shall keep confidential and not disclose and shall take all reasonable security precautions to keep confidential and not disclose to any person other than

to its officers, employees, consultants or professional advisors whose province it is to know, any Confidential Information of another party disclosed to or obtained by it in connection with this Agreement.

17.2 PTC shall only disclose the Confidential Information to those of its Staff who need to know it strictly for the purposes of the Programme and the administration of the Award, provided that they are bound by confidentiality and non-use obligations in respect of such Confidential Information and are first made aware of PTC's confidentiality obligations towards the Trust.

17.3 If PTC considers it necessary for the purpose of the Programme to disclose the Confidential Information to employees, officers, students, visiting academics, contractors, sub-contractors, independent consultants or Third Parties who are not members of PTC's Staff employed on the Programme, then before any such disclosure takes place PTC shall procure that each of the persons concerned are bound by confidentiality and non-use obligations in respect of such Confidential Information and are first made aware of PTC's confidentiality obligations towards the Trust.

17.4 The Exploiting Party shall be entitled to disclose any Confidential Information of PTC or Confidential Information generated during the Programme if it is reasonably necessary or desirable to do so in order to protect, Develop or Exploit the Programme Intellectual Property and/or Products.

17.5 Without prejudice to Clause 17.1, and save in the case of publication in which case the provisions of Clause 15 shall apply, the Parties shall each use

reasonable endeavours to keep details of any Programme Inventions confidential pending filing of a patent application claiming such Programme Invention.

17.6 Clause 17.1 above shall not apply to:

(a) information which is or was already known to the receiving party at the time of disclosure under this Agreement, as shown by the receiving party's written records, without any obligation to keep it confidential;

(b) information which is independently developed by employees of the receiving party who have not had access to the confidential information of the disclosing party;

(c) information which at the time of being disclosed or obtained by the receiving party under this Agreement or at any time thereafter, is published or otherwise generally available to the public other than due to default by the receiving party of its obligations hereunder;

(d) the disclosure of information by the Trust for the purposes of publishing summary details of awards made by the Trust;

(e) the disclosure of information for the purpose of registering a clinical trial on a national or international clinical trial register or on the Trust's clinical trial register or for the purpose of patient recruitment with respect to a clinical trial;

(f) the disclosure to a Party's professional advisers or to the Trust's Site Visit Group of information reasonably required to be disclosed for purposes relating to this Agreement.

17.7 Each Party shall ensure that all Staff, personnel and Third Parties to whom confidential information of the other party is disclosed are informed of the provisions of Clauses 15 (Publications), 16 (Announcements) and this Clause 17 (Confidentiality).

18. WARRANTIES AND INDEMNITIES

18.1 The Trust warrants that:

(a) it has the requisite authority to enter into this Agreement; and

(b) it has full power and authority to assume all of its obligations under this Agreement.

18.2 PTC represents and warrants to the Trust on the Commencement Date and immediately prior to the payment by the Trust to PTC of any Tranche (or installment thereof) that (subject to any matters fairly and accurately disclosed in the Disclosure Letter):

(a) it has the requisite authority to enter into this Agreement;

(b) it has full power and authority to assume all of its obligations under this Agreement;

(c) the Agreement has been duly authorised, executed, and delivered by PTC and is a valid, binding, and legally enforceable obligation of PTC;

(d) no consent, approval, authorisation, or order of any court or governmental agency or body is required for the consummation of the transactions contemplated by this Agreement;

(e) the execution, delivery, and performance of this Agreement will not result in a breach or violation of, or constitute a default under, any statute, regulation, or other law or agreement or instrument to which it is a party or by which it is bound, or any order, rule, or regulation of any court or governmental agency or body having jurisdiction over it or any of its properties;

(f) There have been no amendments to the Treasury Policy since PTC submitted a copy of the Treasury Policy (in English) to the Trust as part of the Application.

(g) PTC's current Treasury Policy is attached at Schedule 7 or has been provided to the Trust in accordance with Clause 2.14 and accepted by the Trust in writing.

(h) PTC is in compliance with the current Treasury Policy accepted by the Trust in writing.

(i) to the best of its knowledge and belief:

(i) PTC is the legal and beneficial owner of, or has appropriate license to, all right, title and interest in and to the Background Intellectual Property necessary for performance of the Programme, and will be the legal and beneficial owner of, or procure appropriate license or option rights to, all right, title and interest in and to the Programme Inventions and Programme Intellectual Property;

(ii) no member of the PTC Group has granted any Third Party any right in respect of the Programme Inventions or Programme Intellectual Property (other than in accordance with the terms of this Agreement), and has not charged or encumbered and will not charge or encumber any of the same except as may be explicitly authorised pursuant to this Agreement;

(iii) so far as PTC is aware, no Third Party has made unauthorised use of any Background Intellectual Property, nor threatened to do so;

(iv) so far as PTC is aware, none of the activities of any member of the PTC Group undertaken by prior to the date on which the warranties are given or which will be undertaken pursuant to the Programme relating to the Background Intellectual Property infringe, or have been alleged to infringe, the Intellectual Property of any Third Party;

(v) the Background Intellectual Property and Programme Intellectual Property are not subject to any claim, opposition, attack, assertion or other arrangements of whatever nature which may impugn upon the use, validity, enforceability or ownership of any such Intellectual Property, and there are no grounds or other circumstances which may give rise to the same;

(vi) From the Commencement Date forward, no member of the PTC Group has itself nor through any of its Staff disclosed to any Third Party (other than consistent with Clause 17) any Confidential Information and/or Know-How relating to the Programme;

(vii) Other than as required by law (including any other contracts with academic collaborators and government entities or in connection with the use of government funds) no person has, or will have, the right to call for the assignment or grant of the licence to it of any of the Background Intellectual Property and the Programme Intellectual Property under any option, grant, funding award or other agreement, nor is there any conditional or unconditional agreement or circumstance whereby such a right may arise;

(viii) no person has any right or claim to any payment or other compensation in respect of the use or exploitation of the Background Intellectual Property or the Programme Intellectual Property; and

(ix) there are no outstanding or potential claims against any member of the PTC Group under any contract or for employee compensation under applicable legislation in relation to the Background Intellectual Property nor is PTC aware of any reason why any such claims may be made in relation to the Programme Intellectual Property.

18.3 Except as expressly provided in this Agreement, neither Party gives any warranties or makes any representations with respect to any of the Programme Intellectual Property and/or Background Intellectual Property or any Products derived from them, or their fitness for any purpose, or that any material produced or supplied by any Party and any processes or techniques used, proposed or recommended by any Party will not infringe any patent or other Intellectual Property of any person in any country.

18.4 Subject to Clause 18.6 below, the Trust's maximum liability in aggregate to PTC arising out of this Agreement shall not exceed the Award Amount.

18.5 Except in circumstances of fraud or wilful misconduct by a Party or its Affiliates, no Party nor any of its Affiliates shall be liable to another Party or any Affiliate of another Party for special, indirect, incidental or consequential damages, whether in contract, warranty, negligence, tort, strict liability or otherwise, arising out of any breach of or failure to perform any of the provisions of this Agreement.

18.6 Nothing in this Agreement shall limit the liability of any Party in respect of:

(a) personal injury or death arising out of that Party's negligence or wilful misconduct, or

(b) fraud or wilful misconduct or fraudulent misrepresentation.

18.7 PTC shall be responsible for and indemnify and keep fully indemnified the Trust and its Affiliates, officers, servants, agents, sub-licensees and sub-sub-licensees (collectively the "**Trust Indemnified Parties**" and each a "**Trust Indemnified Party**") against any and all liability, loss, damage, cost or expense ("**Losses**") incurred or suffered by such Trust Indemnified Party as a result of any claim by a Third Party arising directly out of the Programme and/or the Development, use, promotion, marketing, sale, Exploitation or distribution of the Programme Intellectual Property and/or Products by, or on behalf of, PTC, except to the extent such Losses result from the negligence or intentional misconduct of the Trust Indemnified Party.

19. DISPUTE RESOLUTION

19.1 Any question, difference or dispute which may arise concerning the construction meaning or effect of this Agreement or concerning the rights and liabilities of the Parties hereunder or any other matter arising out of or in connection with this Agreement shall first be submitted to the Director of the Technology Transfer Division of the Trust and the General Counsel of PTC (or their designees) (the "**Senior Officers**"), who may call on others to advise them as they see fit.

19.2 If the Senior Officers are unable to resolve the dispute pursuant to Clause 19.1 within [**] Business Days of the date on which the matter is referred to them, such dispute may be referred by either Party for resolution by an independent chartered accountant (an "**Expert**") to be appointed (in default of

nomination by agreement between the Trust and PTC) by the President for the time being (or next available senior officer) of the Institute of Chartered Accountants in England and Wales. The following provisions shall govern the appointment of the Expert:

(a) The Expert shall prepare a written decision and give notice (including a copy) of the decision to the Parties within a maximum of [**] Business Days of the matter being referred to him.

(b) If the Expert dies or becomes unwilling or incapable of acting, or does not deliver the decision within the time required by Clause 19.2 then:

(i) either Party may apply to the president of the Institute of Chartered Accountants in England and Wales to discharge the Expert and to appoint a replacement Expert with the required expertise; and

(ii) this Clause 19.2 shall apply in relation to the new Expert as if he were the first Expert appointed.

(c) The Parties shall be entitled to make submissions to the Expert including oral submissions and shall provide each Party with a copy of any such submissions and additionally shall provide (or procure that others provide) the Expert with such assistance and documents as the Expert reasonably requires for the purpose of reaching a decision.

(d) To the extent not provided for by this Clause 19, the Expert may, in his reasonable discretion, determine such other procedures to assist with the conduct of the determination as he considers just or appropriate.

(e) Each Party shall, with reasonable promptness, supply each other with all information and give each other access to all documentation and personnel as each other reasonably requires to make a submission under this Clause 19.

(f) The Expert shall act as an expert and not as an arbitrator. The Expert shall determine any dispute, which may include any issue involving the interpretation of any provision of this Agreement, his jurisdiction to determine the matters and issues referred to him or his terms of reference. The Expert's written decision on the matters referred to him, if accepted by the Parties, shall be final and binding in the absence of manifest error or fraud; provided, however that either Party in its sole discretion may decline to accept the Expert's written decision and instead refer the dispute to arbitration pursuant to Clause 19.3.

(g) Each Party shall bear its own costs in relation to the Expert. The Expert's fees and any costs properly incurred by him in arriving at his determination (including any fees and costs of any advisers appointed by the Expert) shall be borne by the Parties equally or in such other proportions as the Expert directs.

19.3 If the procedure under Clauses 19.1 and 19.2 should fail to resolve the question, difference or dispute (including any question regarding the existence, validity or termination of this Agreement) the Parties agree to proceed to binding arbitration. Unless otherwise agreed by the Parties, the arbitration will be take place in London, England, according to the rules of the London Court of International Arbitration ("**LCIA Rules**"), which LCIA Rules are deemed to be incorporated by reference into this clause, except to the extent such rules are inconsistent with this Clause 19.3. The Parties shall bear their own costs of counsel and other professional advisers in such arbitration, regardless of outcome, and the Parties shall share equally in the cost of the arbitration. The arbitration will be conducted by one (1) arbitrator who shall be reasonably acceptable to the Parties and who shall be appointed in accordance with LCIA Rules. If the Parties are unable to select an arbitrator, then the arbitrator shall be appointed by the LCIA. Any arbitrator chosen hereunder shall have educational training and industry experience sufficient to demonstrate a reasonable level of scientific, financial, medical and industry knowledge relevant to the particular dispute. If the question, difference or dispute relates to existence, validity or termination of this Agreement, then the arbitrator shall resolve the question, difference or dispute, and the arbitration shall be conducted according to LCIA Rules. If the question, difference or dispute relates to any other matter under this Agreement, then the arbitration shall be conducted according to the following rules:

(a) Within [**] Business Days after the selection of the arbitrator, each Party shall submit to the arbitrator and the other Party a proposed resolution of the dispute that is the subject of the arbitration, together with any relevant evidence in support thereof (the "**Proposals**").

(b) Within [**] Business Days after the delivery of the last Proposal to the arbitrator, each Party may submit a written rebuttal of the other Party's Proposal and may also amend and re-submit its original Proposal.

(c) The Parties and the arbitrator shall meet within [**] Business Days after the Parties have submitted their Proposals, at which time each Party shall have [**] to argue in support of its Proposal. The Parties shall not have the right to call any witnesses in support of their arguments, nor compel any production of documents or take any discovery from the other Party in preparation for the meeting.

(d) Within [**] Business Days after such meeting, the arbitrator shall select one of the Proposals so submitted by one of the Parties as the resolution of the dispute, but may not alter the terms of either Proposal and may not resolve the dispute in a manner other than by selection of one of the submitted Proposals.

(e) If a Party fails to submit a Proposal within the initial [**] Business Day time frame set forth in the first sentence of Clause 19.3(a), the arbitrator shall select the Proposal of the other Party as the resolution of the dispute. Any time period set forth in this Clause 19.3may be extended by mutual agreement of the Parties.

19.4 The results of an arbitration pursuant to Clause 19.3 shall be binding and enforceable against the Parties in any court of competent jurisdiction, and the Parties hereby consent to the jurisdiction of the English courts for such purpose.

19.5 Notwithstanding the foregoing provisions of Clause 19.3, either Party will have the right to seek interim or provisional relief in any court of competent jurisdiction as may be available to such Party under the laws and rules applicable in such jurisdiction, including a temporary restraining order, preliminary injunction or other interim equitable relief, concerning a dispute either prior to or during arbitration under Clause 19.3, if necessary to protect the interests of such Party or to preserve the status quo pending final arbitration.

20. **DURATION AND TERMINATION**

20.1 This Agreement shall commence on the Commencement Date and shall continue for whichever is the longer of:

(a) the term of the funding under this Agreement and, if applicable, any further funding granted by the Trust in connection with or as a result of the Programme;

(b) the period that the Programme takes to complete;

(c) the last to expire of the Programme Patents;

(d) the expiry of any agreement entered into for the exploitation of the Programme Intellectual Property or the Background Intellectual Property; or

(e) the expiry of any payment obligation relating to the exploitation of the Programme Intellectual Property or the Background Intellectual Property.

20.2 Each Party ("**Terminating Party**") shall have the right to terminate this Agreement forthwith at any time upon giving written notice of termination to the other Party (the "**Defaulting Party**"), upon the occurrence of any of the following events:

(a) the Defaulting Party commits a breach of a material obligation set out in this Agreement which is not capable of remedy;

(b) the Defaulting Party commits a breach of a material obligation set out in this Agreement which is capable of remedy but has not been remedied within [**] Business Days of the receipt by it of a notice from the Terminating Party identifying the breach and requiring its remedy;

(c) the Defaulting Party is unable or admits inability to pay its debts as they fall due, suspends making payments on any of its debts or, by reason of actual or anticipated financial difficulties commences negotiations with one or more of its creditors with a view to rescheduling any of its indebtedness;

(d) a proposal is made or a nominee or supervisor is appointed for a composition in satisfaction of the debts of the Defaulting Party or a scheme or voluntary arrangement of its affairs within the meaning of the relevant bankruptcy or insolvency laws, or the Defaulting Party enters into any composition or voluntary arrangement for the benefit of its creditors, or proceedings are commenced in relation to the Defaulting Party under any law, regulation or procedure relating to the re-construction, deferment or re-adjustment of all or substantially all of the Defaulting Party's debts;

(e) the Defaulting Party takes any action, or any legal proceedings are started whether by a Third Party or not, for the purpose of the winding up or dissolution of the Defaulting Party, other than for a solvent reconstruction or amalgamation;

(f) the appointment of a liquidator, trustee, receiver, administrative receiver, receiver and manager, interim receiver custodian, sequestrator, administrator or similar officer, in respect of all or a substantial part of the assets of the Defaulting Party;

(g) an effective resolution being passed for the winding-up or entering into administration (whether out of court or otherwise) of the Defaulting Party;

(h) a distress, execution or other legal process being levied against all or substantially all of the assets of the Defaulting Party, and not being discharged or paid out in full within [**] Business Days of the commencement of each process; or

(i) the occurrence in respect of the Defaulting Party of any event in any jurisdiction to which it is subject having an effect similar to that of any of the events referred to in Clauses 20.2(c) to 20.2(h) above.

20.3 In addition, the Trust shall be entitled to terminate this Agreement by notice in writing to PTC, such termination to take effect as specified in the notice, if:

(a) During the Programme Term, PTC fails to comply with any of the Conditions and where such non-compliance is capable of remedy, PTC has not remedied it within [**] Business Days of the receipt by it of a notice from the Trust identifying the non-compliance and requiring its remedy;

(b) During the Programme Term, the Site Visit Group recommends termination of the Programme in accordance with Clause 6 and PTC fails to correct any identified failings within the applicable time period under Clause 6.3;

(c) PTC ceases or threatens to cease to carry on all or a substantial part of its business or operations necessary for the completion of its obligations under this Agreement;

(d) PTC takes any action, or omits to take any action, the consequences of which, in the reasonable opinion of the Trust, would be incompatible with or have an adverse effect:

(i) on the Trust's charitable objectives or reputation; or

(ii) on the ability of PTC to comply with its respective obligations under this Agreement; and/or

(e) PTC enters into transactions involving any of the Programme Intellectual Property and/or Background Intellectual Property without the prior written consent of the Trust, including, without limitation, assigning or otherwise transferring any Programme Intellectual Property or

Background Intellectual Property or any interest in such Intellectual Property to an Affiliate or Third Party and/or creating any new security or increasing any existing security over any of the Programme Intellectual Property and/or Background Intellectual Property (other than netting or set-off arrangements entered into in the ordinary course of PTC's banking or financing arrangements for the purpose of netting debit and credit balances; or any lien arising by operation of law and in the ordinary course of business).

20.4 If the during the Programme Term the Principal Investigator ceases to be involved with the Programme, ceases to be employed by or provide services to PTC, ceases to carry out research at premises controlled by PTC, or is prevented through illness or injury from promptly fulfilling his obligations under this Agreement, the Trust shall consult with PTC to ascertain whether the Programme or its progress will be jeopardised by such event. If in the reasonable opinion of the Trust:

(a) such event will jeopardise the Programme or its progress, and the Parties after good-faith negotiations are unable to agree on a replacement Principal Investigator, the Trust may terminate this Agreement by written notice (provided, that such determination and termination by the Trust shall be final and binding and shall not be subject to the dispute resolution or arbitration procedures set forth in Clause 19); or

(b) the Programme has reached a stage such that the services of the Principal Investigator are not key to the completion of the Programme, the Trust and PTC shall negotiate in good faith any amendments necessary to this Agreement so as to enable the satisfactory completion of the Programme within a reasonable time.

20.5 In the event that PTC undergoes a Change of Control, PTC shall give the Trust prompt notice of such Change of Control.

(a) During the Programme Term, if in the Trust's reasonable opinion, the Change of Control would have an adverse effect on, or be incompatible with the Trust's charitable objectives or PTC's ability to fulfil its obligations under the Agreement, the Trust may in its absolute discretion, terminate the Agreement by serving written notice of termination on PTC.

(b) Following the Programme Term, the surviving entity following such Change of Control shall, within [**] days of such Change of Control, confirm in writing to Trust its intentions to continue to meet its obligations under this Agreement, and meet with Trust to present its plans for Exploiting the Programme Intellectual Property and commercializing Products.

(i) If no Product resulting from the Programme is being tested in Phase 1 or later trials or has received Marketing Approval, and if following the meeting contemplated by Clause 20.5(b), in the Trust's reasonable opinion, the Change of Control would have an adverse effect on, or be incompatible with the Trust's charitable objectives or the ability of PTC's successor in interest to fulfil its obligations under the Agreement, the Trust may in its absolute discretion, terminate the Agreement by serving written notice of termination on such successor in interest. For clarity, if any Product resulting from the Programme is being tested in Phase 1 or later trials, and following the meeting contemplated by Clause 20.5(b), the Trust shall not have any termination rights under this Clause 20.5(b)(i), but the Trust and PTC's successor in interest shall negotiate in good faith for a resolution of the issues raised by the Trust, and any failure to reach an agreement on such resolution within a reasonable time period may be referred in the Trust's sole discretion to the dispute resolution and arbitration procedures specified in Clause 19.

(ii) If PTC's successor in interest fails to provide the written notice or refuses to hold the meeting contemplated in Clause 20.5(b), then the Trust may in its absolute discretion, terminate the Agreement by serving written notice of termination on PTC's successor in interest.

21. **EFFECT OF TERMINATION**

21.1 Termination of this Agreement howsoever arising shall be without prejudice to the rights and duties of any Party accrued prior to termination. Except as may be otherwise provided in this Clause 21, the Clauses in this Agreement which expressly have effect after or notwithstanding termination (including without

limitation Clauses 1, 2.10, 2.11, 2.13, 4, 8, 9 10, 11, 12, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, 28, 29 and 30) shall continue to be enforceable notwithstanding termination.

21.2 Upon termination prior to the end of the Programme, PTC shall return all funding received from the Trust under this Agreement which is unspent at the date of termination (after deduction of costs and non-cancellable commitments incurred prior to the date of termination).

21.3 On termination of this Agreement by the Trust in accordance with Clauses 20.2 or 20.5(b)(ii); PTC shall, so far as it is able to do so without violating legal requirements or breaching contractual obligations that existed prior to the event giving grounds for termination:

(a) for no consideration assign all of its rights in the Programme Intellectual Property to the Trust or a Third Party nominated by the Trust;

(b) for no consideration procure that any licences of Programme Intellectual Property granted to PTC shall be assigned to the Trust or a Third Party nominated by the Trust or sub-licensed to the Trust or a Third Party nominated by the Trust on a world-wide, perpetual basis. Such sub-licenses shall be: (a) non-exclusive to complete the Programme and exclusive in relation to Development and Exploitation; and (b) free of charge and royalty free.

(c) upon request from the Trust and at no charge to the Trust, provide such assistance to the Trust as the Trust may reasonably require to assist in the assignment or sub-licensing of the rights in the Programme Intellectual Property or any licences pursuant to this Clause 21.3 and/or in the closure of the Programme;

(d) upon request from the Trust:

(i) grant to the Trust as requested by the Trust a world-wide, royalty free, perpetual, non-exclusive licence to use any and all of the PTC Background Intellectual Property owned or sub-licensable by PTC or any member of the PTC Group and required for further research in accordance with the Programme and/or Development and Exploitation of Programme Intellectual Property; and

(ii) discuss in good faith a worldwide, non-exclusive licence to use any and all of the PTC Background Intellectual Property owned or sub-licensable by PTC or any member of the PTC Group for additional research, Development and Exploitation;

(e) provide to the Trust with all laboratory notebooks and other records relating to the Programme Intellectual Property and the Programme Books and Records;

(f) as requested by the Trust, carry out a hand over of the Programme to the Trust or wind down the Programme for a reasonable period of time, such period not to exceed [**] months following termination; and

(g) return all equipment acquired by PTC using the Trust Award.

21.4 On termination of this Agreement by the Trust in accordance with Clauses 20.3, 20.4, 20.5(a), or 20.5(b)(i), PTC shall meet with Trust to in good faith to address mitigation of any harm to Trust resulting from PTC's actions giving rise to the termination right, and any failure to reach an agreement on such resolution within [**] days may be referred in the Trust's sole discretion to the dispute resolution and arbitration procedures specified in Clause 19. For clarity, this Clause 21.4 shall not be interpreted to limit Trust's ability to seek additional remedies available under this Agreement or otherwise at law with respect to the events giving rise to the applicable termination right.

21.5 On termination of this Agreement by PTC in accordance with Clause 20.2, notwithstanding any other provision of this Agreement, all of the Trust's rights under Clauses 2, 3, 4, 5, 6, 7, 8, 9, 10, 11, 12, 13, 14 and 15, as well as any rights to receive payments pursuant to Schedule 6, shall be extinguished and of no further force and effect. For clarity, this Clause 21.5 shall not be interpreted to limit PTC's ability to seek additional remedies available under this Agreement or otherwise at law with respect to the events giving rise to the applicable termination right.

22. WAIVER

22.1 Neither Party shall be deemed to have waived any of its rights or remedies under this Agreement unless the waiver is expressly made in writing and signed by a duly authorised representative of that Party. In particular, no delay or failure of a Party in exercising or enforcing any of its rights or remedies under this Agreement shall operate as a waiver of those rights or remedies nor shall any single or partial exercise or enforcement of any right or remedy by a Party preclude or impair any other exercise or enforcement of that right or remedy by that Party.

23. ENTIRE AGREEMENT/VARIATIONS

23.1 This Agreement, together with the Application and any agreement entered into pursuant to the Agreement constitutes the entire agreement and understanding between the Parties relating to the subject matter hereof and together they supersede and replace all prior drafts, previous understandings, arrangements, representations or agreements, whether in writing or oral, between the Parties relating to the subject matter of this Agreement.

23.2 No variation, amendments, modification or supplement to this Agreement shall be valid unless and until it is made in writing and signed by a duly authorised representative of each of the Parties.

24. ASSIGNMENT

24.1 Except for a Change of Control in compliance with Clause 20.5, PTC shall not without the prior written consent of the Trust assign, transfer, convey or declare a trust over this Agreement or make any other disposition (whether in whole or in part) of any of its rights and obligations hereunder to any Third Party.

25. SEVERANCE OF TERMS

25.1 If the whole or any part of this Agreement is or becomes or is declared illegal, invalid or unenforceable in any jurisdiction for any reason (including both by reason of the provisions of any legislation and also by reason of any court or competent authority which either has jurisdiction over this Agreement or has jurisdiction over any of the Parties):

(a) In the case of the illegality, invalidity or un-enforceability of the whole of this Agreement it shall terminate only in relation to the jurisdiction in question; or

(b) In the case of the illegality, invalidity or un-enforceability of part of this Agreement that part shall be severed from this Agreement in the jurisdiction in question and that illegality, invalidity or un-enforceability shall not in any way whatsoever prejudice or affect the remaining parts of this Agreement, which shall continue in full force and effect.

25.2 If in the reasonable opinion of any Party any severance under this Clause 25 materially affects the commercial basis of this Agreement, the Parties shall discuss, in good faith, ways to eliminate the material effect.

26. COSTS

26.1 Each Party shall bear its own legal costs, legal fees and other expenses incurred in the preparation and execution of this Agreement.

27. FURTHER ASSURANCES

27.1 Each Party shall perform such acts and execute such documents as may be reasonably required for securing to or vesting in another Party the rights agreed to be granted to it under or pursuant to this Agreement.

28. **NOTICES**

28.1 Any notice to be given pursuant to this Agreement shall be in writing in the English language and shall be delivered by international courier, by registered, recorded delivery or certified mail (postage prepaid) or by facsimile confirmed by registered, recorded delivery or certified mail (postage prepaid) to the address or facsimile number of the recipient Party set out below or such other address or facsimile number as a Party may from time to time designate by written notice to the other Parties. Any notice by facsimile shall be confirmed by the sender sending a confirmatory copy of the notice by registered, recorded delivery or certified mail (postage prepaid).

Address of PTC

PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080
United States

Fax No: +1 (908) 222-1128

for the attention of: Legal Department

With an email copy to legal@ptcbio.com

Address of the Trust

Technology Transfer Division
The Wellcome Trust Limited
215 Euston Road
London NW1 2BE

Fax No: +44 (0) 20 7611 8857

for the attention of: [**]

with a copy to: [**]

28.2 Any notice given pursuant to this Clause 28 shall be deemed to have been received:

(a) in the case of delivery by international courier or sending by certified mail, on the day of receipt, provided receipt occurs on a Business Day of the recipient Party or otherwise on the next following Business Day of the recipient; or

(b) in the case of facsimile, on acknowledgement by the recipient facsimile receiving equipment on a Business Day if the acknowledgement occurs before 5:00 pm local time of the recipient Party and in any other case on the following Business Day.

28.3 Any notice that is required in this Agreement may be validly given if transmitted by fax or sent by post in accordance with this Clause 28. Email alone is not a valid method of giving notice under this Agreement.

29. **GENERAL**

29.1 Nothing in this Agreement shall be taken to constitute a partnership between the Parties. Except as specifically provided in this Agreement, none of the Parties shall by reason of this Agreement be empowered to act as agent for any other party nor to pledge the credit of any other party nor shall any Party be held liable for or incur liability in respect of the acts or defaults of any other Party to this Agreement.

29.2 This Agreement may be executed in any number of counterparts and by the Parties on separate counterparts, but shall not be effective until each Party has executed at least one counterpart. Each counterpart shall constitute an original of this Agreement, but all the counterparts shall together constitute one and the same instrument.

29.3 A person who is not a Party has no right under the Contracts (Rights of Third Parties) Act 1999 to enforce or to enjoy the benefit of any term of this Agreement.

30. **GOVERNING LAW**

30.1 This Agreement (and any dispute, controversy, proceedings or claim of whatever nature arising out of this Agreement or its formation) shall be governed by and construed in accordance with the laws of England. The Parties irrevocably submit to the exclusive jurisdiction of the Courts of England provided that nothing in this clause shall prevent any Party from seeking injunctive relief in any court of competent jurisdiction in respect of a breach or threatened breach of Clause 17 (Confidentiality).

IN WITNESS whereof the Parties or their duly authorised representatives have executed this Agreement on the date hereinbefore written.

SCHEDULE 1

THE PROGRAMME

SDD 02/06

INVITED FINAL APPLICATIONS
FOR A SEEDING DRUG
DISCOVERY STRATEGIC AWARD

APPLICATION FOR A SEEDING DRUG DISCOVERY STRATEGIC AWARD

PLEASE READ THE WELLCOME TRUST'S GRANT CONDITIONS (www.wellcome.ac.uk/fundingpolicy)
AND INFORMATION FOR APPLICANTS (http://www.wellcome.ac.uk/assets/wtx027225.doc)
IN CONJUNCTION WITH THIS APPLICATION FORM.

WELLCOME TRUST DATA PROTECTION STATEMENT

Information that you supply to the Wellcome Trust in connection with this Application (which includes all information sent to the Wellcome Trust that relates to your application, or, in the event of an award, relates to that award) will be used to process your Application and for the purposes of audit and/or evaluation. It may also be disclosed to external peer reviewers, some of whom may be based outside the EEA. Your personal data will be stored by or on behalf of the Wellcome Trust, and/or organisations connected with it, in accordance with the Data Protection Act 1998. Where we fund in partnership with other organisations your personal data may also be disclosed to and processed by the partner(s) involved. The Wellcome Trust may publish basic details of successful awards (e.g. on its website or in its Annual Report) and/or anonymise your personal data for research and statistical purposes. The Wellcome Trust may also release details of successful awards (including your name and employing Institution, the project title, and the scientific abstracts and lay summaries of the research) into the public domain (e.g. via the internet or via publicly accessible databases). The Wellcome Trust may contact you about other award schemes and initiatives that may be of interest to you, or for your views on its funding schemes and application processes. Please contact the Wellcome Trust if you have any questions about the protection of your personal data.

UNDERTAKINGS

1. I confirm that I (and all those providing personal information in the application) have read and understood the Wellcome Trust Data Protection statement above.

2. To the best of my knowledge, the information provided in this application is accurate and complete.

3. I have read the conditions under which grants are awarded and, if a grant is made, I agree to abide by them.

4. The necessary facilities will be made available to conduct this research, and will continue to be available for the duration of the Wellcome Trust's award.

5. During the period of any funding, I will promptly inform the Wellcome Trust of any material changes to any details provided in this application.

Signature of Principal Applicant	[ILLEGIBLE]	**Date:**	8/25/11
Signature of Coapplicant (1)	[ILLEGIBLE]	**Date:**	8/25/11
Signature of Coapplicant (2)		**Date:**	
Signature of Drug Discovery Advisor	[ILLEGIBLE]	**Date:**	8-25-11
Signature of Head of Technology Transfer Office/Group	[ILLEGIBLE]	**Date:**	8-25-11

Front page

SDD 02/06

INVITED FINAL APPLICATIONS
FOR A SEEDING DRUG
DISCOVERY STRATEGIC AWARD

For and on behalf of the Institution:

Signature of Secretary of Institution/Finance Officer/Company Official: /s/ Mark Boulding **Date:** 8/25/11

Position: SVP & General Counsel **Company/Institution:** PTC Therapeutics, Inc.

Q1 Applicants	Principal Applicant	Coapplicant (1)	Coapplicant (2)	Coapplicant (3)	Technology Transfer Officer
Surname	Josyula	Branstrom			Mankoff
Forenames	Vara Prasad V.N.	Arthur			Don
Title (Dr etc.)	Dr	Dr			Mr

Q2 **Title of project:** (no more than 220 characters)

Lead Optimisation and development of novel bacterial DNA synthesis inhibitors for the treatment of nosocomial infections caused by multi-drug resistant Gram-negative bacteria

Q3 **Company name and address or department name and address at administering institution:**

PTC Therapeutics, Inc., 100 Corporate Court, South Plainfield, NJ 07080

Q4 **Type of Translation Award requested:** SEEDING DRUG DISCOVERY

Q5 **Period for which support is sought:**(state in months) 30 months

Q6 **Proposed start date:** (dd/mm/yy) 01/01/12

Principal Applicant

Name	Vara Prasad V.N. Josyula	Telephone numbers:	
Contact address	PTC Therapeutics, Inc 100 Corporate Court South Plainfield, NJ 07080 USA	Day	[**]
		Mobile	[**]
		Fax.	[**]
		e-mail	[**]

Coapplicant (1)

Name	Arthur Branstrom	Telephone numbers:	
Contact address	PTC Therapeutics, Inc 100 Corporate Court South Plainfield, NJ 07080 USA	Day	[**]
		Mobile	[**]
		Fax,	[**]
		e-mail	[**]

Coapplicant (2)

Name		Telephone numbers:	
Contact address		Day	
		Mobile	
		Fax.	
		e-mail	

Technology Transfer Officer

Name	Don Mankoff	Telephone numbers:	
Contact address	PTC Therapeutics, Inc	Day	[**]

	100 Corporate Court South Plainfield, NJ 07080 USA	Mobile	
		Fax.	[**]
		e-mail	[**]

Drug Discovery Advisor

Name	Joseph M. Colacino	Telephone numbers:	
Contact address	PTC Therapeutics, Inc 100 Corporate court South Plainfield, NJ 07080 USA	Day	[**]
		Mobile	[**]
		Fax.	[**]
		e-mail	[**]

Contact details

If a company please provide the following:

Company number:	1-908-222-7000
Date and place of incorporation:	March 31, 1998, Delaware
Shared capital:	
Authorised:	Preferred Stock – 156,995,095, Common Stock – 26,000,000
Issued:	Preferred – 154,728,267, Common – 130,586

Details of Project

Registered holders (name, number and type):

Credit Suisse First Boston Equity Partners,
L.P. and affiliates
HBM BioVentures (Cayman) Ltd.
Private Life BioMed AG
Alstertor Private Life GmbH & Co. KG
Vulcan Ventures Inc. and affiliates
Delphi Ventures and affiliates
Celgene International Inc.
The Bay City Capital Fund III, L.P. and affiliates
Birchrnere Ventures III LP
Novo A/S
Healthcap 1999 KB and affiliates
Pfizer International LLC
The Column Group, LP
Novartis BioVentures Ltd.
Genavent Partners LP
Gilead Palo Alto, Inc. (f/k/a/ CV Therapeutics, Inc.)
Amgen SF, LLC and affiliates
Manufacturers Life Insurance
General Electric Capital Corporation
Deutsche Bank Nominees (Jersey) Limited a/c HAML
Pictet Funds (LUX)
Coller International Partners IV Limited
CDIB Biotech USA Investment, Co., Ltd.
POSCO BioVentures I, L.P.
Agron Ltd. Inc
Tenue Business S.A
Ptaza St Petersburg Holdings Ltd
Marbre Services Limited
Frederick Frank
Security Pacific Finance, Ltd.
Minglewood Management Ltd.
Edward Spencer-Churchill
Wolvercote Investments Limited (BVI)
Leman Management Nominees

	The Swanson Family Fund, Ltd. Stronghold Capital Ltd (formerly Three Crowns Capital (Cayman) Ltd.) Mintz Levin Investments LP The Board of Trustees of the Leland Stanford Junior University (DAPER I) HSBC Trustee (C.I.) Limited Great China Trading Company Tichenor Ventures LLC
Registered Office:	N/A
Directors	CEO – Stuart W. Peltz, Ph.D. CFO – William Baird General Counsel/Secretary – Mark Boulding

Secretary:	Mark Boulding
Accounting reference date:	13/12/2010
Previous source of funding and amount:	[**]
Cash in bank and other investments:	[**]
Average monthly expenditure:	[**]
Board of Directors:	Michael Schmertzler, Axel Bolte, Soren Carlsen, Ph.D., Carl Goldfischer, M.D., Allan Jacobson, Ph.D., Michael Kranda, Deepa Pakianathan, Ph.D., Stuart W. Peltz, Ph.D., David P. Southwell
Scientific advisory Board:	Allan Jacobson, Ph.D., Eric N. Jacobsen, Ph.D., Paul A. Marks, M.D., Robert Schneider, Ph.D., Marvin Wickens, Ph.D., Joseph Puglisi, Ph.D.
Number of Employees:	171 Full time employees, 5 part-time employees.

Please enclose a copy of the current Business Plan Executive Summary with application.

Q7 TIME SPENT BY APPLICANTS ON RESEARCH

(a) How many hours per week do the Principal Applicant and Coapplicant(s) spend on research?

Principal Applicant	Coapplicant 1	Coapplicant 2	Coapplicant 3	Coapplicant 4
[**]	[**]			

(b) How many hours per week will be spent on this project by the Principal Applicant and Coapplicant(s)?

Principal Applicant	Coapplicant 1	Coapplicant 2	Coapplicant 3	Coapplicant 4
[**]	[**]			

Q8 RELATED APPLICATIONS

(a) Is this or a related application currently being submitted elsewhere? YES x NO o

If yes, to which organisation? National Institute of Allergy and infectious Disease's

By what date is a decision expected? (dd/mm/yy) 01/05/2012

(b) Has this, or a similar, application been submitted elsewhere over the past year? YES o NO x

If yes, to which organisation?

What was the result?

(c) Is this application a resubmission or has it been previously considered under another Wellcome Trust scheme? YES x NO x

If yes, when was it originally considered?

Please give the Wellcome Trust's reference number:

Briefly state how this application differs from the original (no more than 250 words)

Q9 SUMMARY OF PROPOSED RESEARCH INCLUDING KEY GOALS

(a) For scientifically qualified assessors: (no more than 200 words)

The goal of this proposal is to optimise and develop structurally novel compounds inhibiting clinically validated targets for the treatment of infections caused by Gram-negative pathogens. The key differentiating factor of PTC's current lead compounds is their antibacterial activity against multi-drug resistant (MDR) Gram-negative pathogens unlike the available treatments targeting the bacterial DNA synthesis pathway. The PTC compounds demonstrate broad spectrum antibacterial activity that includes *E. coli, A. baumannii, K. pneumoniae, H. influenzae, M. catarrhalis* and S. *aureus*. In addition to monotherapeutic utilisation, PTC's compounds are amenable to combination therapy with fluoroquinolones, as synergy with ciprofloxacin has been observed against a wide variety of pathogens. While both classes of compounds target DNA synthesis, this synergistic effect indicates PTC's compounds interact with DNA gyrase and topoisomerase IV in a manner different from that of fluoroquinolones. We propose to identify the specific binding motif of the PTC compounds to the topoisomerase-DNA-inhibitor ternary complex through X-ray crystallography. The PTC compounds compounds also exhibited *in vivo* efficacy in murine models of systemic infection. We propose parallel and complementary approaches, which includes medicinal chemistry and structure-based drug design, with the goal of improving antibacterial activity, efficacy and pharmaceutical properties in the identification of a Development Candidate (DC). Once the DC is identified, we will develop a process for its large-scale synthesis and acceptable formulation to initiate IND-enabling safety studies for submission of an IND application.

(b) For lay readers: (no more than 200 words)

This research proposal outlines a plan for developing a new class of molecules for the treatment of Gram-negative bacterial infections that are refractory to current drugs. These infections are caused by bacteria that have developed resistance to almost all known treatments. There is a great need to develop new drugs to combat the diseases caused by these dangerous strains. We have already identified general features of our molecules that are essential for their antibacterial activity. This has led to molecules that successfully kill a wide variety of drug-resistant bacteria, including the recently discovered New Delhi strain (*E. coli* NDM-1) and MRSA. We have accumulated data that demonstrate our molecules interact with their target in a different manner than do available antibiotics and can be used in combination with Cipro® or other fluoroquinolones. We propose parallel and complementary approaches, which includes medicinal chemistry and structure-based drug design, with the goal to improve antibacterial activity, efficacy, and pharmaceutical properties in the identification of a Development Candidate (DC). Once the DC is identified, we will develop a process for the large-scale synthesis and acceptable formulation to initiate safety studies required for submission of an Investigational New Drug application.

Q10 What is the total requested cost from the Trust?

Funding dependent milestones			Cumulative Cost		
Cost of Milestone Period 1 (M1):			$	1,092,000	
Cost of Milestone Period 2 (M2):	$	1,974,000	$	3,066,000	=M1+M2
Cost of Milestone Period 3 (M3):	$	1,967,000	$	5,033,000	=M1+M2+M3

Q11 Define the primary objective of the proposal and briefly summarise the project work plan. (Maximum 1 page)

NOTICE OF PROPRIETARY INFORMATION: The information in this application constitutes trade secrets or information that is commercial or financial and confidential or privileged. It is furnished to the Wellcome Trust in confidence with the understanding that such information shall be used or disclosed only for evaluation of this application.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 32 pages were omitted. [**]

Q22 BIOGRAPHIES OF ADVISORS AND STEERING GROUP

Neil Almstead, PhD, Senior Vice President, Research and CMC, PTC Therapeutics Dr. Almstead joined PTC in 2000, and is responsible for PTC's efforts in research and manufacturing. Dr. Almstead joined PTC from Procter & Gamble (P&G) where he was directly involved in the areas of inflammatory diseases and oncology. At P&G he was project leader for both the Matrix Metalloproteinase and Map kinase inhibitor projects. Dr. Almstead has coauthored more than 75 publications and patents pertaining to the design and synthesis of lead candidate compounds for genetic disorders, oncology and inflammatory diseases. After receiving his Ph.D. in Organic Chemistry from the University of Illinois at Urbana-Champaign under the direction of Professor Scott Denmark, Dr. Almstead was a postdoctoral associate at the University of Basel in Switzerland with Professor Bernd Giese.

Joseph M. Colacino, PhD, Vice President, Drug Discovery, PTC Therapeutics

Dr. Colacino joined PTC in March 2003, and oversees all drug discovery activities across therapeutic areas. He brings to PTC over 14 years of drug discovery experience from Eli Lilly and Company. At Eli Lilly, he held positions as Research Acquisition Advisor, Director of Virology, and Head of Biology for Infectious Diseases Research. In that capacity, Dr. Colacino initiated and directed research programs in Infectious Diseases and managed a number of internal committees involved in the evaluation of screening methodologies and scientific approaches. Dr. Colacino is widely published and serves on the editorial boards of Antimicrobial Agents and Chemotherapy, Antiviral Chemistry and Chemotherapy, and Progress in Drug Research and has been an expert reviewer for Journal of Virology, Virology, Journal of Antimicrobial Chemotherapy, Antiviral Research, Virus Research, and Proceedings of the National Academy of Science. Dr. Colacino received his Ph.D. in Virology from the Cornell Graduate School of Medical Sciences, New York. He is currently the president of the International Society for Antiviral Research.

Eric N. Jacobsen, PhD, Sheldon Emery Professor of Organic Chemistry, Harvard University

Dr. Jacobsen is a member of PTC Therapeutics Scientific Advisory Board; in addition, he is a consultant for Merck & Co., Inc. and Amgen, inc. Dr. Jacobsen has received numerous awards for his endeavors, including the NSF Presidential Young Investigator Award (1990), the Packard Fellowship (1991), the Camille and Henry Dreyfus Teacher-Scholar Award (1992), the Alfred P. Sloan Foundation Fellowship (1992), the Cope Scholar Award (1993), the Fluka "Reagent of the Year" Prize (1994), the Thieme-IUPAC Prize in Synthetic Organic Chemistry (1996), the Baekeland Medal (1999), the ACS Award for Creativity in Synthetic

Organic Chemistry (2001), election to the American Academy of Arts & Sciences (2004) , the ACS HC Brown Award for Creativity in Synthetic Methods (2008) and election to the National Academy of Sciences (2008). Dr. Jacobsen received his Ph.D. in Organometallic Chemistry from the University of California, Berkeley.

Q23 PREVIOUS APPLICATIONS TO THE WELLCOME TRUST

(a) Is this the Principal Applicant's first application to the Wellcome Trust? YES x NO o

(b) Give details of all previous applications to the Wellcome Trust over the last five years. This information should be provided for the Principal Applicant and all Coapplicant(s). Please include name of grant holder, grant number (if known) and, if application was successful, the amount and period of award.

Curriculum Vitae of Applicant(s)

Q24 CURRENCY REQUESTED

(a)	What currency is being used for the costings in this application?	US$
(b)	Is the chosen currency your local currency?	Yes
(c)	What is your local currency?	US$
(d)	Please specify the exchange rate with your local currency that has been used to provide the costings in this application.	NA

(e) Please state clearly the reasons for requesting costs in the chosen currency (no more than 150 words)

PTC does not have a presence outside the US and as such most of our costs are in US$. We do not hold any other currency in our bank accounts and do not maintain any bank accounts outside the US. PTC always requests that contracts with foreign entities be in US$ so that we can avoid foreign exchange risk when possible.

Q25 SUMMARY OF FINANCIAL SUPPORT REQUESTED

PLEASE NOTE: VAT ON CONTRACT RESEARCH AGREEMENTS IS ALLOWABLE. THESE AGREEMENTS ARE SUBJECT TO NORMAL PROCUREMENT PROCEDURES.

Duration of grant (state in months): 30 months

	Total cost
(a) Salaries	[**]
(b) Materials and consumables	[**]
(c) Animals	[**]
(d) Equipment	[**]
(e) Miscellaneous	[**]
GRAND TOTAL	**$ 5,033,000 US$**

Financial details

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED

(a) Salaries

Please refer to guidance notes and definition of terms for further details. Expand table as necessary. Budget in US$

Post no.	Staff category	Name (if known)	Starting salary	Grade/ Scale	increment date (dd/mm)	Start date (dd/mm/yy)	EFFORT ON PROJECT: Period on project (months)	EFFORT ON PROJECT: % of full time	London Allowance	Total of other allowances	Employer's contribution	Total cost on grant

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [**]

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED

(a) Salaries

Please refer to guidance notes and definition of terms for further details. Expand table as necessary. Budget is in US$.

Post no.	Staff category	Name (if known)	Starting salary	Grade/ Scale	Increment date (dd/mm)	Start date (dd/mm/yy)	EFFORT ON PROJECT Period on project (months)	EFFORT ON PROJECT %of full time	London Allowance	Total of other allowances	Employer's contributions	Total cost on grant

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [**]

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)

Expand table as necessary.

		Costs
(b) Materials and consumables (description)		
Chemistry Supplies		
Solvents		[**]
Reagents		[**]
Chemical Intermediates		[**]
Glassware		[**]
Biology Supplies		
Reagents		[**]
Cell/Tissue Culture		[**]
Assay Kits		[**]
Bacterial strains		[**]
	Subtotal	**[**]**
(c) Animals		
Total purchase cost		[**]
Total maintenance cost		[**]
Total procedures cost		[**]
	Subtotal	**[**]**

The table below should be duplicated for **each different species.**

(i)	**Animal species to be used, and strain if relevant**	[**]
(ii)	**Source of supply**	[**] [**] [**]
(iii)	**Purchase**	
	Purchase price per animal	[**] [**]
	Total number of animals to be purchased	[**]

		[**]
	Total purchase cost	[**] [**] [**]
(iv)	**Maintenance**	
	Total number of animals to be maintained	[**]
	Total number of weeks' maintenance required	[**] [**] [**] [**] [**]
	Cost per animal per week	[**] [**] [**]
	Total maintenance cost	[**]

(v)	**Experimental procedures**
	Types of procedure(s)
	Cost per procedure(s)
	Total procedures cost

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)

(d) Equipment

Please provide contact details for the Institution's Director of Procurement/Head of Purchasing (or equivalent).

Name: Tel:

Address: E-mail:

(i) Request for equipment. Expand table as necessary.

Type of equipment	Equipment specification	Preferred manufacturer/ supplier (if known)	Duration & total cost of maintenance contract to be purchased	Number of items	Cost per item	Total cost
					Total:	
					Contribution from other sources:	
					Amount requested:	

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)

(d) Equipment (cont.)

(ii) Request for equipment maintenance. Expand table as necessary.

Maintenance of existing Wellcome Trust-funded equipment

The Wellcome Trust will only consider providing maintenance funds for equipment more than five years old if the applicant can demonstrate it is cost-effective to do so.

Details of equipment/facility	Wellcome Trust grant reference number and start/end dates of original award	Date of purchase	Start/end dates of any current maintenance contract, length and total cost	% of time/hours of use for this project	Maintenance cost requested based on time used for this project

(iii) Request for access charges. Expand table as necessary.

Access charges

Details of equipment/facility	Original source of funding (provide Wellcome Trust grant reference number if applicable)	Standard access charge per hour/day	% of time/hours of use for this project	Access charge requested based on time used for this project

Q26 DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED (cont.)

Expand table as necessary.

(e) Miscellaneous (description)	Costs
Collaborations	[**]
CMC	[**]
IND Tox	[**]
Subtotal	[**]

Q27 ACCESS TO RADIATION SOURCES

(a) Will the proposed research require access to either the Synchrotron Radiation Source (SRS) at Daresbury or the European Synchrotron Radiation Facility (ESRF) at Grenoble? YES o NO x

If yes, please complete the table below, providing details of beam time requested and scheduling information (anticipated usage must be specified in whole days).

Synchrotron	Station	Special requirements (single bunch, other specify)	Total number of days	Number of days per annum				
				Year 1	Year 2	Year 3	Year 4	Year 5

(b) Please justify the stations and beam time requested (no more than 500 words).

(c) Will the proposed research require access to a neutron source? YES o NO x

If yes, complete Q26 (a) and (b) above indicating that it is a neutron source that is required, and Q25 (d)(iii) Access charges, detailing the costs required.

Q28 REASONS FOR SUPPORT REQUESTED

In this section, justify;

(a) Staff requested specifying their roles, responsibilities and location, if appropriate, with respect to the proposed project (no more than 700 words)

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of three pages were omitted. [**]

Justification of costs requested

SDD 02/05

Q29 FULL ECONOMIC COSTING (UK applicants only)

The Wellcome Trust would like to monitor the full economic cost of research proposals. If your institution is calculating the full economic costs of this proposal, the table below should be completed.

Please note that the Wellcome Trust will not fund the full economic cost of research and the actual costs sought from the Wellcome Trust should be detailed in the 'DETAILS OF FINANCIAL SUPPORT AND RESOURCES REQUESTED' section of the form.

This information is being gathered for monitoring purposes only and will have no bearing on the peer review and decision-making process for your application.

(a) Does the host institution use TRAC or an alternative methodology validated by the UK Research Councils to calculate full economic costs? YES o NO o

(b) If yes, please complete the following table:

	Full Economic Cost (£)	Contribution requested from the Wellcome Trust (£)
Directly Incurred Costs		
Staff		
Travel and subsistence		
Other costs		
Equipment		
Subtotal		
Directly Allocated Costs		
Principal Applicant salary costs		
Coapplicant salary costs		
Estates costs		
Other directly allocated costs		
Subtotal		
Indirect Costs		

TOTAL _____ _____

Administration

Q30 RESEARCH INVOLVING HUMAN PARTICIPANTS, BIOLOGICAL SAMPLES AND PERSONAL DATA

(a) Does your project involve human participants? YES o NO x

If yes, refer to notes.

(b) Will personal data be used? YES o NO x

(c) Will your project involve use of biological samples? YES o NO x

(d) Please state by whom the project will be, or has been, ethically reviewed, and specify any other regulatory approvals that have been, or will be, obtained.

NA

(e) In the course of your project:

(i) Do you propose to use facilities within the National Health Service (NHS)? YES o NO x

(ii) Does your research involve patients being cared for by the NHS? YES o NO x

(iii) If the answer is yes to (i) or (ii) above, please indicate which organisation has agreed to be the sponsor for the project under the Research Governance Framework for Health and Social Care, published by the Department of Health in England or the corresponding departments in Northern Ireland, Scotland or Wales.

Please note that the Wellcome Trust cannot act as sponsor.

NA

(f) If your project involves a clinical trial:

(i) Please state whether it is covered by The Medicines for Human Use (Clinical Trials) Regulations. YES o NO o

(ii) Please indicate which organisation has agreed to be the sponsor for the project.

Please note that the Wellcome Trust cannot act as sponsor.

NA

Q31 EXPERIMENTS ON ANIMALS

Please note, this question is mandatory for all applications for funding that propose research using animals. Applications may be referred to the National Centre for the Replacement, Refinement and Reduction of Animals in Research (NC3Rs) for review. Where animal work is sub-contracted, this question must be completed by the organization conducting the animal studies.

(a) Do your proposals involve the use of animals or animal tissue? YES x NO o

(b) Do your proposals include procedures to be carried out on animals in the UK which require a Home Office licence? YES o NO x

If yes, refer to notes.

(c) Does the institution where the animal work is to be carried out hold a certificate of designation under the Animals (Scientific Procedures) Act 1986? YES x NO o

(d) Do your proposals involve the use of animals or animal tissue outside the UK? YES x NO o

If yes, refer to notes.

(e) If your project does involve the use of animals, what would be the severity of the Mild procedures? x

Moderate x

Substantial x

(f) Please provide details of any procedures of substantial or moderate severity (no more than 250 words).

[**].

(h) Why is the species to be used the most appropriate? (no more than 250 words)

[**].

Q32 RISKS OF RESEARCH MISUSE

(a) It is the responsibility of institutions in receipt of Wellcome Trust funding to ensure that any risks that research could be misused for harmful purposes are managed in an appropriate manner.

Please tick the box to confirm that you have considered whether your proposed research could generate outcomes that could be misused for harmful purposes. x

(b) If you have identified any tangible risks of this type, please briefly describe these risks and the steps that you and your institution will take to manage them (no more than 250 words).

We have no indication that an antibiotic targeting the inhibition of bacterial DNA synthesis would have potential for misuse.

Q33 LOCATION OF RESEARCH

(a) Will the research project be undertaken in a Wellcome Trust Clinical Research Facility? YES o NO x

If yes, please specify:

(b) Will the research project be undertaken in the Wellcome Trust Sanger Institute or a Wellcome Trust Centre? YES o NO x

If yes, please specify:

Please provide a letter of support from the Director of the Centre/Clinical Research Facility specified.

Q34 CONSULTANCIES AND EQUITIES

Do any of the applicants have consultancies or any equity holdings in companies or other organisations that might have an interest in the results of the proposed research? YES x NO o

If yes, refer to notes and give brief details (no more than 200 words).

As determined in his/her capacity as a PTC Therapeutics, Inc. employee

Q35 TREASURY POLICY (MANDATORY)

(a) It is essential that applicants provide this treasury policy information with their application. Applications will not be taken forward for consideration unless this information is given. Please refer to the Seeding Drug Discovery Information for Applicants for details of what is required.

Seeding Drug Discovery strategic awards are managed through a funding agreement that provides funding in advance of each milestone based upon the agreed budget necessary for delivery of defined objectives. With advance funding, award funds will be placed with your bank until they are used and the Wellcome Trust therefore has an interest in understanding how and where they will be held.

Please either (i) provide a copy of your organisation's treasury policy (in English) with your application; or (ii) insert details of your organisation's treasury policy in the space below.

Please note, this will not have any impact on consideration of the merits of your application, but will allow us to ensure funding can be released in a timely manner, if your application is successful. The Wellcome Trust must be promptly notified of any changes to your treasury policy between application and award (and throughout the duration of the award).

Treasury Policy is included as: Attachment C_PTC Treasury Policy

(b) Provide the name and contact details of the person within your organisation responsible for your organisation's treasury policy ("Treasury Policy Contact") for us to contact in the event we require further information or treasury policy updates:

Name:	[William Baird, III]
Position:	[Chief Financial Officer]
Address:	[100 Corporate Court. S. Plainfield, NJ 07080, USA]
Phone number:	[1-908-912-9159]
Email:	[wbaird@ptcbio.com]

SUBJECT CLASSIFICATION

1. SYSTEMS AND PROCESSES

Choose one primary (compulsory) and up to three secondary (optional).
Infection

2. DISEASE

Choose one primary (compulsory) and up to three secondary (optional).

Bacterial

3. DISCIPLINE

Choose one primary (compulsory) and up to three secondary (optional).

Chemistry
Microbiology – Bacteriology
Pharmacology/Pharmacy

4. TECHNIQUE

Choose up to three (optional).

Cell culture
Crystallography
Mass spectrometry

5. OTHER IDENTIFIER

Choose up to six (optional).

Antibiotic
Antibiotic resistance

6. Tick all relevant boxes (compulsory).

BASIC	o	
CLINICAL		x
TROPICAL		o
VETERINARY	o	
TRANSLATION	x	

COLLABORATION
ON A GRANT FORM

Reference Number:

Collaborators, i.e. scientific/medical colleagues, who are associated with a research proposal and named in the body of the application, but are not Coapplicants, are asked to complete this form.

Name of grant applicant:

Department and institution:

Name of collaborator:

Full address:

Title of research project:

Collaboration form

Extent and nature of collaboration:

(A brief paragraph providing details of:

· The role and contribution of the collaborator, with an indication of the time the collaborator will spend on the project.

· Any reagents the collaborator will provide. Please indicate if there are any Intellectual Property issues or restrictions arising from Material Transfer Agreements.)

I confirm that I am willing to collaborate as stated above with on this research project

Signed: Date:

(if more than one copy of this form is required, duplicate as necessary)

EQUAL OPPORTUNITIES
MONITORING FORM
CONFIDENTIAL

The Commission for Racial Equality and the Equal Opportunities Commission recommend collecting data to monitor the fairness of selection decisions. There is no obligation to provide this information but the Wellcome Trust would be grateful if the Principal Applicant would complete this form to assist with this process. On receipt, this form will be separated from the completed application form. The information provided will be regarded as strictly confidential and will be held on a secure database; it will not be shown to anybody involved in the processing of the application. The Wellcome Trust will anonymise these data (i.e. remove the applicant's name) when using them for statistical and research purposes.

Name: [**]

1. Sex: [**]

2.Date of birth: [**]

3. Ethnic origin: [**]

4. Disability: [**]

Equal opportunities monitoring form

APPENDIX

Appendix A

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [**]

APPENDIX

Appendix B

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of nine pages were omitted. [**]

APPENDIX

Appendix C

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of five pages were omitted. [**]

SCHEDULE 2

CONDITIONS

1. Compliance with PTC's obligations with respect to the formation and operation of the RSG in accordance with Clause 5;

2. Engagement and involvement of an independent industry adviser to the RSG with relevant experience in accordance with Clause 5.2(a)(ii);

3. Timely submissions of reports on Programme progress pursuant to Clause 5.7;

4, Co-operation with Site Visit Group prior to and during visits in accordance with Clause 6;

5. Timely consultation with the IPMG on patent strategy and patent prosecution in accordance with Clause 9.

6. Compliance with the Grant Conditions and any other agreements between PTC and the Trust relating to the Programme;

7. Seeking and maintaining protection of Background Intellectual Property in accordance with Clause 8.4; and

8. Compliance with PTC's treasury policy (such policy to be acceptable to the Trust) in accordance with Clause 2.2.

SCHEDULE 3

MILESTONES, MILESTONE DATES AND TRANCHES

Milestone number	Instalments Payments of Milestone amounts (can include portions of more than one relevant milestone)	Instalment Payment Date
1	[**]	Within [**] Business Days of the Commencement Date
	[**]	Within [**] months of the Commencement Date
2	[**]	Within [**] Business Days of the achievement of Milestone 1, in accordance with Clause 2.3(b)
	[**]	Within [**] months of the achievement of Milestone 1, in accordance with Clause 2.3(b)
3	[**]	Within [**] Business Days of the achievement of Milestone 2, in accordance with Clause 2.3(b)
	[**]	Within [**] months of the achievement of Milestone 2, in accordance with Clause 2.3(b)
4	[**]	Within [**] Business Days of the achievement of Milestone 3, in accordance with Clause 2.3(b)
TOTALS	$ 5,033,000	

Milestone number	Milestone Description	Milestone Date
1	[**]	[**] months after Commencement Date
2	[**]	[**] months after Commencement Date
3	[**]	[**] months after Commencement Date

SCHEDULE 4

Costs of Goods

The Parties acknowledge and agree that in the event an unforeseen cost arises that is not specified below, they shall negotiate in good faith whether such cost is an allowable cost.

For the purpose of calculating the cost of Product a standard costing approach is to be applied in accordance with the Accounting Standard. The following types of expenses shall be included:

(i) direct materials;

(ii) direct labour;

(iii) indirect manufacturing costs;

(iv) quality assurance, and

(v) certain variances as set out in paragraph 5 below, but not including other Production costs, as identified below.

Each of these categories of expenses are further specified below.

In any event, the Cost of Goods shall include all cost elements appropriate under the Accounting Standard.

1. Direct Materials

Materials used in the manufacturing processes that are traced directly to the completed Product, such as:

- Inert raw materials or excipients
- Active substances/ingredients
- Packaging components such as bottles, caps, labels, etc.

2. Direct Labour

The cost of employees engaged in Production activities that are directly identifiable with Product costs. This shall exclude supervision, which is included in indirect labour, and Production support activities such as inspection, plant and equipment maintenance labour, and material handling personnel. Direct labour cost includes:

- Base pay, overtime, vacation and holidays, illness, personal time with pay and shift differential.
- Cost of employee fringe benefits such as health and life insurance, payroll taxes, welfare, pension and profit sharing.

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3. Indirect Manufacturing Costs

Costs for plant and equipment are to be applied to standard costs taking normal capacity utilization as a reference.

Costs which are ultimately allocated to Product based on standard direct labour hours of the operating departments. These costs include:

- Indirect Production Labour - salaries of employees engaged in Production activities which are not classified as direct labour, including supervision, clerical, etc.
- Costs of Direct Labour - employees not utilized for the manufacturing of Product such as training and general duties.
- Indirect Materials - supplies and chemicals which are used in the manufacturing process and are not assigned to specific Products but are included in manufacturing overhead costs. Includes supplies for which direct assignment to Products is not practical.
- Utilities - expenses incurred for fuel, electricity and water in providing power for Production and other plant equipment and waste disposal.
- Maintenance and Repairs - amount of expense incurred in-house or purchased to provide services for plant maintenance and repairs of facilities and equipment.
- Other Services - purchased outside services and rentals such as the cost of security, ground maintenance, etc.
- Depreciation - of plant and equipment utilizing the straight-line method of calculation.
- Insurance - cost of comprehensive and other insurance necessary for the safeguard of manufacturing plant and equipment.
- Taxes - expense incurred for taxes on real and personal property (manufacturing site, buildings and the fixed assets of equipment, furniture and fixtures, etc.) If manufacturing site includes other operations (marketing, R&D, etc.), taxes are allocated to manufacturing on the basis of total real and personal property.
- Cost of manufacturing, service departments - such as (where applicable):
  - Packaging Engineering
  - Manufacturing Maintenance
  - Industrial Engineering
  - Receiving and Warehousing
  - Purchasing and Accounting

  - Production Scheduling
  - Inventory Management
  - Plant Materials Management

- Central Weigh
- Manufacturing Administration

· Allocated costs of services provided to manufacturing including: (where applicable):

- Cafeteria
- Personnel Operations
- Health and Safety Services
- Division Engineering and Operations Services
- Plant Services (housekeeping)
- Manufacturing Information Systems
- Plant Power
- Office of V.P. Manufacturing

Various bases are used for allocating these costs to manufacturing operating departments including headcount, square feet, metered utilities use, estimated services rendered, EDP computer hours, etc.

4. Quality Assurance Costs

Direct labour and indirect costs for quality assurance departments testing and approving materials used in manufacturing and completed manufacturing batches and finished Products. This includes all manufacturing in-process testing and testing of finished materials. Excluded from Product costs are quality assurance costs related to research and development, stability testing, and other costs customarily excluded from such quality assurance costs.

5. Variance Costs

· Standard Cost of Goods include cost elements which are set at so-called standard costs. They serve as a norm on how much typically a Product costs. Deviations from such standard costs are captured in variances

· Inventory re/devaluation shall mean the gain or loss as a result of the inventory value adjustment due to changes in the standard costs.

· Non-Product related Production costs shall contain technical operations corporate headquarter overhead costs, non Product allocated QA costs, validation costs, directly expensed IT Programme costs, and other costs that cannot be attributed to specific Products.

· Warehousing & distribution costs are costs related to warehousing and distribution activities for finished goods to be shipped to 3rd parties.

· Write-offs are captured for the destruction of Products that cannot be used anymore due to expiration of shelf-life, spoilage in the Production process, and transportation mishaps.

· Third Party royalties are manufacturing and/or supply royalties paid to Third Parties

The following expenses are not included in Production costs:

a) Inventory carrying costs;

b) Regulatory affairs costs;

c) Pilot plant costs, research batches and other similar costs prior to turnover to manufacturing (excluding commercial goods produced by a research facility);

d) Costs incurred by manufacturing for special Programmes to establish and certify new Production processes, batch sizes and Product line improvements, and new vendor certification of equipment and primary materials components;

e) Manufacturing start-up costs and initial one-time extraordinary manufacturing costs incurred prior to plant operation and achievement of a normal Production activity level; including the costs of training, testing, qualification/validation of new equipment and facilities and initial, trial batches;

f) Significant idle capacity is eliminated from factory overhead and Product cost;

g) Finished goods warehousing, shipping and other distribution costs;

h) Product liability and/or business interruption insurance expenses; and

i) Intercompany profit.

In-kind contributions from PTC and the PTC Group

1. Infrastructure: laboratories and office space, including the costs of buildings, rental, fixtures and utilities) at the PTC's facilities in New Jersey, USA (the "**PTC Facilities**") and provision of the services of the PTC FTEs to the extent set out in the Application.

2. Access to existing equipment and laboratory benches within the PTC Facilities.

SCHEDULE 6

REVENUE SHARING TERMS

1) Introduction

a) This Schedule 6 sets out the revenue sharing terms ("**Revenue Sharing Terms**") agreed between the Parties.

b) Each scenario below shall apply based on the description of the scenario.

2) **Scenario 1**: PTC exploits the Programme Intellectual Property on a For-Profit Basis alone (or in collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of worldwide commercialization).

a) PTC shall pay the following stage-based milestones based on multiples of the total Trust Contribution through Regulatory Approval:

i) Milestone triggering events and amounts:

[**]

ii) Worked Example: assumes Trust funds $5 million US and PTC does not elect to defer payment of either the Phase 1 or the Phase 3 milestone:

(1) Phase 1 milestone amount = $[**]

(2) Phase 2 milestone amount = $[**]

(3) Phase 3 milestone amount = $[**]

(4) Regulatory Approval milestone amount = $[**]

(5) Total of all milestone amounts= $[**]

iii) Worked Example: assumes Trust funds $5 million and PTC defers at both Phase 1 and Phase 3:

(1) Phase 1 milestone amount = $[**] (deferred as provided below)

(2) Phase 2 milestone amount = $[**]

(3) Phase 3 milestone amount = $[**] (deferred as provided below)

(4) Regulatory Approval milestone amount = $[**]

(5) Total of all milestone amounts = $[**]

iv) Payment of milestones

(1) Phase 1-Phase 3 milestones shall be payable in equal quarterly installments over expected term of study, with the first installment payment due within [**] Business Days of the milestone triggering event. PTC may by written notice to the Trust elect to defer payment of the Phase 1 milestone until after completion of the first Phase 1 study, in which case PTC shall pay such Phase 1 milestone (including an additional 0.05x due for the deferral option) within [**] year of completion of the Phase 1 study. PTC may by written notice to the Trust elect to defer payment of the Phase 3 milestone until after completion of the first Phase 3 study to be completed in either the USA or the EAA (whichever is the sooner) required for Regulatory Approval (the "**Trigger Phase 3**"), in which case PTC shall pay such Phase 3 milestone (including additional 1x due for the deferral option) within [**] year of completion of the

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Trigger Phase 3 study. For clarity, if deferred, the payment of Phase 3 milestone is due regardless of outcome of Phase 3 trial(s); provided, that the Trust agrees to accept alternative consideration, such as equity, in the event a cash payment after a Phase 3 trial failure would place PTC in financial distress.

(2) For clarity, milestones are payable only for the first Product to reach the applicable milestone.

(3) The Regulatory Approval milestone shall be payable on the [**]; provided, however, that the Trust will consider in good faith payment of the Regulatory Approval milestone in installments if PTC revenue from all products at the time of Regulatory Approval is less than $[**].

b) In addition to any milestones payable in accordance with the preceding section, PTC shall also pay royalties on Net Sales of Products, on a Product-by-Product basis; provided, that such royalties shall only be payable in the event the Trust Contribution represents at least [**]% of the proposed $5million

US funding amount, and shall be scaled proportionately in the event the Trust Contribution is greater than [**]% but less than 100% of the proposed $5 million US funding amount:

i) Royalty scale based on Net Sales of Product:

(1) First $[**]%

(2) Next $[**]%

(3) Next $[**]%

(4) Next $[**]%

(5) Over $[**]%

ii) Royalties payable shall be payable on a country-by-country basis until the longer of (a) the expiration last Valid Claim of a patent in the applicable country or region covering the Product, or (b) the expiration of marketing exclusivity of a Product in the applicable country or region based on applicable law.

3) **Scenario 2**: PTC exploits the Programme Intellectual Property on a For-Profit Basis through outlicensing of a Product to a Third Party on a worldwide, exclusive basis prior to Regulatory Approval.

a) The Parties shall hold an economic stake in the Product ("**Base Shares**") calculated as of outlicensing effective date based on their respective economic contributions.

i) On the Commencement Date, PTC begins with $15 million Base Shares, and the Trust with zero.

ii) As the Trust pays the proposed ~$5 million US funding amount over the Programme Term, the Trust's Base Share shall increase proportionately. By way of example, (a) [**].

iii) Following the Programme Term, PTC's ownership of Base Shares shall increase proportionately based on the continuing economic contribution of PTC itself. By way of example, [**].

b) All consideration attributable to outlicensing to a Third Party (other than debt at arm's length interest rates or bona fide research funding) shall be divided between PTC and the Trust according to relative Base Share ownership at the time of such outlicensing. By way of example, [**].

c) For clarity, once outlicensing under this scenario has occurred, then the milestones provided for in scenario 1 shall no longer apply following the effective date of the outlicense; provided, that if a milestone trigger event occurred prior to the outlicense

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but installment payments are ongoing, PTC must complete such milestone payments.

d) For clarity, neither PTC nor the Third Party gaining the outlicense shall make any royalty payments to the Trust under this scenario.

e) License or access payments to Third Parties for enabling technologies required, in the good faith judgment of PTC, to develop and commercialize a Product shall be counted in the calculation of Base Shares under this scenario; provided, however, that such payments shall not include license or access payments made with respect to the composition of matter or method of use of those active ingredient(s) in the Product that incorporate, comprise or are derived from the Programme Intellectual Property.

4) **Scenario 3**: PTC exploits the Programme Intellectual Property on a For-Profit Basis by retaining development/commercialization rights to Product in some regions of the World or with respect to some uses of the Product (either alone or in a collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of commercialization), and outlicenses the Product on an exclusive basis in other regions of the World or with respect to other uses of the Product.

a) In this scenario, any consideration from outlicensing (other than debt at arm's length interest rates or bona fide research funding) shall be divided between the parties according to Base Shares as of effective date of the outlicense.

b) In addition, following such outlicense, PTC shall pay milestones and royalties based on scenario 1 for those regions of the World or uses of the Product for which it retains rights, subject to the following adjustments:

i) PTC will prepare a written proposal for adjustment to milestones and royalties based on its modeling of the relative values of market share outlicensed vs. market share retained by PTC.

(1) The Trust shall consider PTC's proposal in good faith, and prepare a written counterproposal if it wishes;

(2) The Parties shall negotiate in good faith for reasonable allocation of relative value of markets based on their proposals;

(3) If the Parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

(4) Once the relative value of the markets outlicensed versus the markets retained by PTC is determined, PTC's obligation to make continuing milestone and royalty payments pursuant to Scenario 1 shall be reduced according to relative value of markets outlicensed versus the markets retained. By way of example, if PTC outlicensed [**] of the market value of a Product, then a milestone payment of $[**] owed under scenario 1 would be reduced to a milestone payment of $[**] under this scenario 3, and a [**]% Net Sales royalty under scenario 1 would become a [**]% Net Sales royalty under this scenario 3.

5) **Other Scenarios:**

a) If a situation arises that is not covered by any of the foregoing three scenarios, the parties will negotiate in good faith for an appropriate economic arrangement based on Base Shares.

i) If the parties cannot agree, each party shall prepare a written proposal and accompanying rationale for an appropriate economic arrangement.

ii) The parties shall then negotiate in good faith based on their respective proposals.

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iii) If parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

SCHEDULE 7

PTC's Treasury Policy

PTC THERAPEUTICS
Recommended Investment Policy Guidelines

Date Last Reviewed: March 8, 2007

1. PURPOSE
To establish guidelines for the investment of surplus cash balances. Surplus cash balances are balances in corporate accounts not immediately required for working capital, capital investment, debt repayment, or other outstanding near-term financial obligations.

2. OBJECTIVES
The objectives of the policy are, in order of priority:
2.1 Preservation of capital.
2.2 Fulfillment of liquidity needs.
2.3 Above-market returns versus industry averages.
2.4 Fiduciary control of cash and investments.

3. ELIGIBLE INVESTMENTS
All investments must be U.S. dollar-denominated.
Borrowing for investment purposes is prohibited.
Investment in securities with underlying leverage risk or esoteric structures is prohibited.
3.1 U.S. Treasury bills, notes, and bonds
- 3.1.1 Includes putable, callable, and floating-rate obligations

3.2 U.S. agency debt obligations
- 3.2.1 Includes obligations issued by government-sponsored enterprises
- 3.2.2 Includes putable, callable, and floating-rate obligations

3.3 Corporate debt obligations
- 3.3.1 Rated one of the following
  - 3.3.1.1 A or better by Moody's and Standard & Poor's
  - 3.3.1.2 P1 by Moody's and A1 or better by Standard and Poor's
- 3.3.2 Includes variable-rate demand notes
- 3.3.3 Includes putable, callable, and floating-rate obligations
- 3.3.4 Includes Eurodollar and Yankee debt obligations

3.4 Bank debt obligations
- 3.4.1 Rated one of the following:
  - 3.4.1.1 A or better by Moody's and Standard & Poor's
  - 3.4.1.2 P1 by Moody's and A1 or better by Standard and Poor's.
- 3.4.2 Includes variable-rate demand notes
- 3.4.3 Includes putable, callable, and floating-rate obligations
- 3.4.4 Includes Eurodollar and Yankee debt obligations

3.5 Taxable, tax-exempt, and tax-advantaged municipal debt obligations
- 3.5.1 Rated one of the following:
  - 3.5.1.1 A or better by Moody's and Standard & Poor's
  - 3.5.1.2 MIG1 or VMIG1 by Moody's and SP1 or better by Standard &Poor's
  - 3.5.1.3 P1 by Moody's and A1 or better by Standard & Poor's
- 3.5.2 Includes variable-rate demand notes
- 3.5.3 Includes putable, callable, and floating-rate obligations
- 3.5.4 Tax-exempt and tax-advantaged municipal debt obligations are eligible only when PTC THERAPEUTICS is a tax-paying entity

3.6 Money market funds
- 3.6.1 SEC-Registered

- 3.6.2 Maintain a net asset value of $1.00/share
- 3.6.3 Consist of a minimum of $1 billion in assets

3.7 Repurchase agreements
- 3.7.1 Collateralized at a minimum of 102% with one of the following:
  - 3.7.1.1 U.S. Treasury bills, notes, or bonds
  - 3.7.1.2 U.S. agency debt obligations

3.7.2 Collateral may not have maturities in excess of 24 months
3.8 Asset-backed securities
3.8.1 Rated one of the following:
3.8.1.1 AAA by Moody's or Standard & Poor's
3.8.1.2 P1 by Moody's or A1+ by Standard & Poor's
3.8.2 Asset-backed commercial paper rated P1 by Moody's and A1+ by Standard & Poor's

4. CONCENTRATION LIMITS
4.1 There is no limit to the percentage of the portfolio that may be maintained in U.S. Treasury debt obligations, U.S. agency debt obligations, or SEC-registered money market funds.
4.2 With the exception of those investments listed in Section 4.1, no one issuer or group of issuers from the same holding company is to exceed fifteen (15) percent of the book value of the portfolio at the time of purchase.

5. MATURITY LIMITS
5.1 The maximum maturity of individual securities in the portfolio may not exceed twenty four (24) months.
5.2 The weighted-average days to maturity of the portfolio may not exceed twelve (12) months.
5.3 For securities that have put, reset, or expected average maturity dates, the put, reset, or expected average maturity dates will be used, instead of the final maturity dates, for maturity limit purposes.
5.4 The liquidity requirement stated in Section 6. will always take priority over the maturity limits stated in Sections 5.1, 5.2, and 5.3.

6. LIQUIDITY REQUIREMENT
6.1 A minimum of two times the amount of expected monthly cash outflow must be liquid each business day.
6.1.1 Liquidity may be reduced below the amount of two times expected monthly cash outflow upon written notice by PTC THERAPEUTICS
6.1.2 For purposes of determining liquidity, book value will be used.
6.2 The sale of securities prior to maturity is permitted only for managing liquidity and must be pre-approved by PTC THERAPEUTICS for fiduciary control purposes.

7. INVESTMENT PERFORMANCE
7.1 Capital Advisors Group will issue a quarterly investment performance analysis using time-weighted measures.
7.2 A quarterly meeting will be held with the individual appointed by the Board for fiduciary controls, to review performance figures and any updated liquidity needs.

8. CREDIT QUALITY
8.1 Trends for a given company or industry must be reviewed periodically by the investment manager and adjustments in percentage positions made accordingly.
8.2 Should any investment held in PTC THERAPEUTICS's portfolio fall short of prescribed guidelines, immediate notification must be made to the individual appointed by the Board to oversee fiduciary control.

9. MARKETABILITY
9.1 All securities must be purchased through investment banking and brokerage firms of high quality and reputation, with a history of making markets for the securities in which PTC THERAPEUTICS invests.

9.2 In the unlikely event that securities must be sold before their maturity, the securities must be easily remarketed. To accomplish this, the securities must be conventional products with strong name recognition.

10. TRADING GUIDELINES
10.1 Normal investing practice is to reinvest the funds on the day a security matures, to minimize lost interest.
10.2 A daily transaction log is to be maintained and available for review at any time.
10.3 All trading firms must generate a hard copy document for each transaction that is mailed to Capital Advisors Group, Inc. on behalf of PTC THERAPEUTICS.
10.4 Quarterly summaries of PTC THERAPEUTICS's investment holdings and cash usage are to be made available for Board review.

11. CUSTODY
11.1 Assets are to be held in a segregated bank custody account with separate fiduciary documents executed by the bank. Assets shall not be held by any investment manager or securities dealer.

12. FIDUCIARY DISCRETION
12.1 The Chief Financial Officer or other individual appointed by the Board and his/her authorized employees are responsible for securing and managing investments and cash for operations.
12.2 These individuals have full discretion to invest any excess capital subject to strict adherence to these guidelines.
12.3 These guidelines are to be reviewed periodically with the Chief Financial Officer or Chief Executive Officer and revisions made consistent with objectives set forth herein.

SCHEDULE 8

Amendment to Application

Criteria for the selection of [**] advanced lead optimisation candidates and DC.

Property	Target value [**]	Target value [**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

[**]	[**]	[**]
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[**] [**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Signed for and on behalf of

PTC THERAPEUTICS, INC.

by its duly authorised representative:

Signature: /s/ Mark E. Boulding

Name: Mark E. Boulding

Title: SVP & General Counsel

Date: 21 December 2011

Signed for and on behalf of

THE WELLCOME TRUST LIMITED as
trustee of the Wellcome Trust

by its duly authorised representative:

Signature: /s/ Dr. Richard Seabrook

Name: Dr. Richard Seabrook

Title: Head of Business Development
Technology Transfer

Date:

Signed for and on behalf of

THE WELLCOME TRUST LIMITED as
trustee of the Wellcome Trust

by its duly authorised representative:

Signature: /s/ Dr. A.E. Bianco

Name: Dr. A.E. Bianco

Title: Director of Technology Transfer
The Wellcome Trust

Date:

Execution Copy

LOAN AND SECURITY AGREEMENT

THIS LOAN AND SECURITY AGREEMENT (this "**Agreement**") dated as of September 21, 2009 (the "**Effective Date**") among **OXFORD FINANCE CORPORATION**, a Delaware corporation with an office located at 133 North Fairfax Street, Alexandria, Virginia 22314 ("**Oxford**"), as collateral agent ("**Collateral Agent**"), the Lenders listed on Schedule 1.1 hereof and otherwise party hereto from time to time (each a "**Lender**" and collectively, the "**Lenders**"), and **PTC THERAPEUTICS, INC**., a Delaware corporation ("**Borrower**"), provides the terms on which Lenders shall lend to Borrower and Borrower shall repay Lenders. The parties agree as follows:

1 ACCOUNTING AND OTHER TERMS

Accounting terms not defined in this Agreement shall be construed following GAAP. Calculations and determinations must be made following GAAP. Capitalized terms not otherwise defined in this Agreement shall have the meanings set forth in Section 14. All other terms contained in this Agreement, unless otherwise indicated, shall have the meaning provided by the Code to the extent such terms are defined therein.

2 LOAN AND TERMS OF PAYMENT

2.1 Promise to Pay Borrower hereby unconditionally promises to pay each Lender, the outstanding principal amount of all Term Loans advanced to Borrower by such Lender and accrued and unpaid interest thereon and any other amounts due hereunder as and when due in accordance with this Agreement.

2.2 Term Loans.

(a) Availability. Subject to the terms and conditions of this Agreement, the Lenders agree, severally and not jointly, (i) during the First Draw Period, to make term loans to Borrower in an aggregate amount up to Twelve Million Five Hundred Thousand Dollars ($12,500,000.00) according to each Lender's Term A Loan Commitment Percentage as set forth on Schedule 1.1 hereto (such term loans are hereinafter referred to singly as a "**Term A Loan**", and collectively as the "**Term A Loans**") and (ii) during the Second Draw Period, to make additional term loans to Borrower in an aggregate amount up to Twelve Million Five Hundred Thousand Dollars ($12,500,000.00) according to each Lender's Term B Loan Commitment Percentage as set forth on Schedule 1.1 hereto (subject to adjustment as provided on Schedule 1.1) (such term loans are hereinafter referred to singly as a "**Term B Loan**", and collectively as the "**Term B Loans**"; each of the Term A Loans and the Term B Loans is referred to singly herein as a "**Term Loan**", and the Term A Loans and the Term B Loans are referred to collectively herein as the "**Term Loans**"). After repayment, no Term Loan may be re-borrowed.

(b) Repayment. Borrower shall make monthly payments of interest only (accrued at the applicable interest rate as set forth in Section 2.3 of this Agreement) on each Term Loan commencing on the first (1st) Payment Date following the Funding Date of such Term Loan, and continuing on the Payment Date in each of the following (i) five (5) consecutive months with respect to the Term Loan A and (ii) six (6) consecutive months with respect to the Term Loan B. Commencing on the Amortization Date in respect of a Term Loan, and continuing on the Payment Date of each month thereafter, Borrower shall make consecutive equal monthly payments of principal and interest, in arrears, on such Term Loan to each Lender, as calculated by Collateral Agent based upon: (1) the principal amount of such Lender's Term Loan, (2) the effective rate of interest, as determined in Section 2.3(a), and (3) a repayment schedule equal to thirty (30) months. All unpaid principal and accrued interest with respect to each Term Loan is due and payable in full on such Term Loan's Maturity Date. The Term Loans may only be prepaid in accordance with Sections 2.2(c) and 2.2(d).

(c) Mandatory Prepayments. (i) If the Term Loans are accelerated following the occurrence of an Event of Default, Borrower shall immediately pay to Lenders, payable to each Lender in accordance with its respective Pro Rata Share, an amount equal to the sum of: (A) all outstanding principal of the Term Loans plus accrued interest thereon through the prepayment date, (B) the Final Payment, (C) the Prepayment Fee, plus (D) all other sums, that shall have become due and payable, including Lenders' Expenses, if any, and interest at the Default Rate with respect to any past due amounts.

(ii) In the event Borrower permanently discontinues Borrower's pursuit of active development of Ataluren (also known as PTC124®) for all therapeutic indications (as determined by the Lenders in their reasonable discretion) (the "**PTC124 Discontinuation**"), Borrower will give prompt written notice to Collateral Agent, and the Lenders shall have the right, upon written notice to Borrower, to require Borrower to repay the Term Loans in full, in which case Borrower shall immediately pay to Lenders, payable to each Lender in accordance with its respective Pro Rata Share, an amount equal to the sum of: (A) all outstanding principal of the Term Loans plus accrued interest thereon through the prepayment date (accrued at the applicable interest rate as set forth in Section 2.3 of this Agreement), (B) the Final Payment, (C) an amount equal to fifty percent (50%) of the Prepayment Fee, plus (D) all other sums, that shall have become due and payable, including Lenders' Expenses, if any, and interest at the Default Rate with respect to any past due amounts. Notwithstanding the foregoing, the PTC124 Discontinuation shall not be deemed to have occurred (and the Lenders shall not have the right to require Borrower to repay the Term Loans as a result of the PTC124 Discontinuation) in the event that after the Effective Date Borrower receives a lump sum cash payment(s) (which payment(s) are recognized by Borrower as revenue or equity, or any combination thereof, but not indebtedness) of at least $25,000,000 in the aggregate.

(d) Permitted Prepayment of Loans. Borrower shall have the option to prepay all, but not less than all, of the Term Loans advanced by the Lenders under this Agreement, provided Borrower (i) provides written notice to Collateral Agent of its election to prepay the Term Loans at least fifteen (15) days prior to such prepayment, and (ii) pays to the Lenders on the date of such prepayment, payable to each Lender in accordance with its respective Pro Rata Share, an amount equal to the sum of (A) all outstanding principal of the Term Loans plus accrued interest thereon through the prepayment date (accrued at the applicable interest rate as set forth in Section 2.3 of this Agreement), (B) the Final Payment, (C) the Prepayment Fee, plus (D) all other sums, that shall have become due and payable, including Lenders' Expenses, if any, and interest at the Default Rate with respect to any past due amounts.

(e) Final Payment. Notwithstanding (but without duplication with) the foregoing provisions of this Section 2.2, on the Maturity Date of a Term Loan, Borrower shall pay to each Lender in accordance with its respective Pro Rata Share the Final Payment in respect of such Term Loan.

2.3 Payment of Interest on the Credit Extensions.

(a) Interest Rate. Subject to Section 2.3(b), the principal amount outstanding under the Term Loans shall accrue interest at a fixed per annum rate equal to the Basic Rate, calculated by Collateral Agent on the Funding Date of the Term Loans, which interest shall be payable monthly in accordance

with Sections 2.2(b) and 2.3(e). Interest shall accrue on each Term Loan for the day on which the Term Loan is made, and shall accrue on a Term Loan, or any portion thereof, for the day on which the Term Loan or such portion is paid.

(b) Default Rate. Immediately upon the occurrence and during the continuance of an Event of Default, Obligations shall bear interest at a rate per annum which is five percentage points (5.00%) above the Basic Rate (the "**Default Rate**"). Payment or acceptance of the increased interest rate provided in this Section 2.3(b) is not a permitted alternative to timely payment and shall not constitute a waiver of any Event of Default or otherwise prejudice or limit any rights or remedies of Collateral Agent.

(c) 360-Day Year. Interest shall be computed on the basis of a 360-day year consisting of twelve (12) months of thirty (30) days.

(d) Debit of Accounts. Each Lender may debit any of Borrower's deposit accounts, including the Designated Deposit Account, for principal and interest payments or any other amounts Borrower owes

such Lender under the Loan Documents when due. These debits shall constitute a loan payment and not constitute the exercise of a right of set-off.

(e) Payments. Except as otherwise expressly provided herein, all loan payments by Borrower hereunder shall be made to the respective Lender to which such payments are owed, at such Lender's office in immediately available funds on the date specified herein. Unless otherwise provided, interest is payable monthly on the Payment Date of each month. Payments of principal and/or interest received after 12:00 noon Eastern time are considered received at the opening of business on the next Business Day. When a payment is due on a day that is not a Business Day, the payment is due the next Business Day and additional fees or interest, as applicable, shall continue to accrue until paid. All payments to be made by Borrower hereunder or under any other Loan Document, including payments of principal and interest made hereunder and pursuant to any other Loan Document, and all fees, expenses, indemnities and reimbursements, shall be made without set-off, recoupment or counterclaim, in lawful money of the United States and in immediately available funds.

2.4 Secured Promissory Notes. Each Term Loan shall be evidenced by a Secured Promissory Note in the form attached as **Exhibit D** hereto (each a "**Secured Promissory Note**"), and shall be repayable as set forth herein. Borrower irrevocably authorizes each Lender to make or cause to be made, on or about the Funding Date of any Term Loan or at the time of receipt of any payment of principal on such Lender's Secured Promissory Note, an appropriate notation on such Lender's Secured Promissory Note Record reflecting the making of such Term Loan or (as the case may be) the receipt of such payment. The outstanding amount of each Term Loan set forth on such Lender's Secured Promissory Note Record shall be, absent manifest error, a prima facie evidence of the principal amount thereof owing and unpaid to such Lender, but the failure to record, or any error in so recording, any such amount on such Lender's Secured Promissory Note Record shall not limit or otherwise affect the obligations of Borrower hereunder or under any Secured Promissory Note to make payments of principal of or interest on any Secured Promissory Note when due. Upon receipt of an affidavit of an officer of a Lender as to the loss, theft, destruction, or mutilation of its Secured Promissory Note together with a lost security indemnity from the related Lender, Borrower shall issue, in lieu thereof, a replacement Secured Promissory Note in the same principal amount thereof and of like tenor.

2.5 Fees. Borrower shall pay to Collateral Agent (or in the case of clause (b) below, to the Lenders):

(a) Facility Fee. A fully earned, non-refundable facility fee of Three Hundred Twelve Thousand Five Hundred Dollars ($312,500) to be shared between the Lenders pursuant to their respective Commitment Percentages. The facility fee shall be due and payable in two installments as follows: (i) $234,375 of the facility fee shall be due and payable on the Effective Date, and (ii) the remaining $78,125 of the facility fee shall be due and payable on the date on which Borrower achieves positive Phase IIb results for Borrower's Ataluren program (PTC124) for the Duchenne indication, which shall be evidenced by a final written determination to proceed, without further testing or trials, with the filing of a new drug application with the US Food and Drug Administration for Ataluren for the Duchenne indication, which such determination has been made by the joint development committee for Ataluren (which includes Genzyme, Inc. and Borrower) acting unanimously and supported by the findings of the independent safety monitoring board for the Phase IIb Ataluren clinical trial ("**Positive Pivotal Ataluren Data**"), whether or not Borrower draws down the Term B Loans. In the event Borrower does not achieve Positive Pivotal Ataluren Data on or prior to April 30, 2010, the second installment of the facility fee of $78,125 shall not be due and payable.

(b) Final Payment. The Final Payment, when due hereunder, to be paid to the Lenders in accordance with their respective Pro Rata Shares;

(c) Prepayment Fee. The Prepayment Fee, when due hereunder, to be shared between the Lenders in accordance with their respective Pro Rata Shares; and

(d) Lenders' Expenses. All Lenders' Expenses (including reasonable attorneys' fees and expenses for documentation and negotiation of this Agreement) incurred through and after the Effective Date, when due (and in the absence of any other due date specified herein, such Lenders' Expenses shall be due within five (5) days of demand therefor).

3 CONDITIONS OF LOANS

3.1 Conditions Precedent to Initial Credit Extension. Each Lender's obligation to make a Term Loan is subject to the condition precedent that Collateral Agent shall consent to or shall have received, in form and substance satisfactory to Collateral Agent, such documents, and completion of such other matters, as Collateral Agent may reasonably deem necessary or appropriate, including, without limitation:

(a) duly executed original signatures to the Loan Documents to which Borrower is a party;

(b) duly executed original signatures to the Control Agreements with Wachovia Bank and Capital Advisors Group/State Street Bank (collectively, the "Borrower's Account Banks");

(c) duly executed original Secured Promissory Notes in favor of each Lender according to its Commitment Percentage in amounts not to exceed the Term Loans;

(d) the Operating Documents of Borrower and good standing certificates of Borrower certified by the Secretary of State of the State of Delaware as of a date no earlier than thirty (30) days prior to the Effective Date;

(e) good standing certificates certified by the Secretary of State of the State of New Jersey as of a date no earlier than thirty (30) days prior to the Effective Date to the effect that Borrower is qualified to transact business in such State;

(f) duly executed original signatures to the completed Borrowing Resolutions for Borrower;

(g) Collateral Agent shall have received certified copies, dated as of a recent date, of financing statement searches, as Collateral Agent shall request, accompanied by written evidence (including any UCC termination statements) that the Liens indicated in any such financing statements either constitute Permitted Liens or have been or, in connection with the initial Credit Extension, will be terminated or released;

(h) a landlord's consent executed in favor of Collateral Agent in respect of each of Borrower's facilities located in South Plainfield, New Jersey;

(i) a bailee's consent executed in favor of Collateral Agent in respect of Borrower's property located in the facilities of Borrower's vendor, Kendle International, in Hamilton County, Cincinnati, Ohio;

(j) a legal opinion of Borrower's counsel dated as of the Effective Date together with the duly executed original signatures thereto;

(k) evidence satisfactory to Collateral Agent that the insurance policies required by Section 6.5 hereof are in full force and effect, together with appropriate evidence showing loss payable and/or additional insured clauses or endorsements in favor of Collateral Agent, for the ratable benefit of the Lenders; and

(l) payment of the fees and Lenders' Expenses then due as specified in Section 2.5 hereof.

3.2 Conditions Precedent to all Credit Extensions. The obligation of each Lender to make each Credit Extension, including the initial Credit Extension, is subject to the following conditions precedent:

(a) timely receipt by the Collateral Agent of an executed Payment/Advance Form in the form of **Exhibit B** attached hereto;

(b) the representations and warranties in Section 5 shall be true, in all material respects on the date of the Payment/Advance Form and on the Funding Date of each Credit Extension; provided, however, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; and provided, further that those representations and warranties expressly

referring to a specific date shall be true, accurate and complete in all material respects as of such date, and no Event of Default shall have occurred and be continuing or result from the Credit Extension. Each Credit Extension is Borrower's representation and warranty on that date that the representations and warranties in Section 5 remain true, accurate and complete in all material respects; provided, however, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; and provided, further that those representations and warranties expressly referring to a specific date shall be true, accurate and complete in all material respects as of such date; and

(c) in such Lender's reasonable discretion, there has not been any Material Adverse Change or any material adverse deviation by Borrower from the most recent business plan of Borrower presented to and accepted by Collateral Agent.

3.3 Covenant to Deliver. Borrower agrees to deliver to Collateral Agent each item required to be delivered to Collateral Agent under this Agreement as a condition precedent to any Credit Extension. Borrower expressly agrees that a Credit Extension made prior to the receipt by Collateral Agent of any such item shall not constitute a waiver by the Lenders of Borrower's obligation to deliver such item, and any such Credit Extension in the absence of a required item shall be made in Collateral Agent's sole discretion.

3.4 Procedures for Borrowing. Subject to the prior satisfaction of all other applicable conditions to the making of a Term Loan set forth in this Agreement, to obtain a Term Loan, Borrower shall notify Collateral Agent (which notice shall be irrevocable) by electronic mail, facsimile, or telephone by 12:00 noon Eastern time three (3) Business Days prior to the date the Term Loan is to be made. Together with any such electronic or facsimile notification, Borrower shall deliver to Collateral Agent by electronic mail or facsimile a completed Payment/Advance Form executed by a Responsible Officer or his or her designee. Upon receipt of a Payment/Advance Form, Collateral Agent shall promptly provide a copy of the same to each Lender. Collateral Agent may rely on any telephone notice given by a person whom Collateral Agent reasonably believes is a Responsible Officer or designee. On the Funding Date, each Lender shall credit and/or transfer (as applicable) to Borrower's Designated Deposit Account, an amount equal to its Term Loan Commitment.

4 CREATION OF SECURITY INTEREST

4.1 Grant of Security Interest. Borrower hereby grants Collateral Agent, for the ratable benefit of the Lenders, to secure the payment and performance in full of all of the Obligations, a continuing security interest in, and pledges to Collateral Agent, for the ratable benefit of the Lenders, the Collateral, wherever located, whether now owned or hereafter acquired or arising, and all proceeds and products thereof. Borrower represents, warrants, and covenants that the security interest granted herein is and shall at all times continue to be a first priority perfected security interest in the Collateral, subject only to Permitted Liens that may have priority as permitted by the terms of this Agreement. If Borrower shall acquire a commercial tort claim (as defined in the Code), Borrower shall promptly notify Collateral Agent in a writing signed by Borrower of the general details thereof (and further details as may be required by Collateral Agent) and grant to Collateral Agent, for the ratable benefit of the Lenders, in such writing a security interest therein and in the proceeds thereof, all upon the terms of this Agreement, with such writing to be in form and substance reasonably satisfactory to Collateral Agent.

If this Agreement is terminated, Collateral Agent's Lien in the Collateral shall continue until the Obligations (other than inchoate indemnity obligations) are repaid in full in cash. Upon payment in full in cash of the Obligations and at such time as the Lenders' obligation to make Credit Extensions has terminated, Collateral Agent, and if appropriate, each Lender shall, at Borrower's sole cost and expense, release its Liens in the Collateral and all rights therein shall revert to Borrower.

4.2 Authorization to File Financing Statements. Borrower hereby authorizes Collateral Agent to file financing statements, without prior notice to Borrower, with all appropriate jurisdictions to perfect or protect Collateral Agent's and each Lender's interest or rights hereunder, including a notice that any disposition of the Collateral, by either Borrower or any other Person, shall be deemed to violate the rights of Collateral Agent and the Lenders under the Code. Such financing statements may indicate the Collateral as "all assets of Debtor" or words of similar effect, or as being of an equal or lesser scope, or with greater detail, all in Collateral Agent's discretion (provided that such financing statements shall specifically refer to those assets excluded from Collateral on Exhibit A hereto). Collateral Agent shall use commercially reasonable efforts to provide Borrower with a copy of all financing statements filed, indicating the jurisdiction and date of filing, promptly after each such filing, provided that failure of Collateral Agent to provide Borrower with such copies or other

information shall not impair the validity or priority of any financing statement or impair or restrict any of the rights and remedies of Collateral Agent and the Lenders under the Loan Documents.

5 REPRESENTATIONS AND WARRANTIES

Borrower represents and warrants as follows at all times unless expressly provided below:

5.1 Due Organization, Authorization: Power and Authority. Borrower and each of its Subsidiaries, if any, are duly existing and in good standing, as Registered Organizations in their respective jurisdictions of formation and are qualified and licensed to do business and are in good standing in any jurisdiction in which the conduct of their business or their ownership of property requires that they be qualified except where the failure to do so could not reasonably be expected to have a material adverse effect on Borrower's business. In connection with this Agreement, Borrower has delivered to Collateral Agent a completed perfection certificate signed by Borrower (the "**Perfection Certificate**"). Borrower represents and warrants that (a) Borrower's exact legal name is that indicated on the Perfection Certificate and on the signature page hereof; (b) Borrower is an organization of the type and is organized in the jurisdiction set forth in the Perfection Certificate; (c) the Perfection Certificate accurately sets forth Borrower's organizational identification number or accurately states that Borrower has none; (d) the Perfection Certificate accurately sets forth Borrower's place of business, or, if more than one, its chief executive office as well as Borrower's mailing address (if different than its chief executive office); (e) Borrower (and each of its predecessors) has not, in the past five (5) years, changed its jurisdiction of formation, organizational structure or type, or any organizational number assigned by its jurisdiction; and (f) all other information set forth on the Perfection Certificate pertaining to Borrower and each of its Subsidiaries is accurate and complete (it being understood and agreed that Borrower may from time to time update certain information in the Perfection Certificate after the Effective Date to the extent permitted by this Agreement). If Borrower is not now a Registered Organization but later becomes one, Borrower shall promptly notify Collateral Agent of such occurrence and provide Collateral Agent with Borrower's organizational identification number.

The execution, delivery and performance by Borrower of the Loan Documents to which it is a party have been duly authorized, and do not (i) conflict with any of Borrower's Operating Documents, (ii) contravene, conflict with, constitute a default under or violate any material Requirement of Law, (iii) contravene, conflict or violate any applicable order, writ, judgment, injunction, decree, determination or award of any Governmental Authority by which Borrower or any of its Subsidiaries or any of their property or assets may be bound or affected, (iv) require any action by, filing, registration, or qualification with, or Governmental Approval from, any Governmental Authority (except such Governmental Approvals which have already been obtained and are in full force and effect), or (v) constitute an event of default under any material agreement by which Borrower or any of its Subsidiaries or their respective properties is bound (other than the Oxford Equipment Financing, which default has been waived by Oxford). Borrower is not in default under any agreement to which it is a party or by which it is bound in which the default could reasonably be expected to have a material adverse effect on Borrower's business.

5.2 Collateral. Borrower has good title to, has rights in, and the power to transfer each item of the Collateral upon which it purports to grant a Lien hereunder, free and clear of any and all Liens except Permitted Liens. Borrower has no Deposit Accounts, Securities Accounts, Commodity Accounts or other investment accounts other than the Collateral Accounts with Borrower's Account Banks or the other investment accounts, if any, described in the Perfection Certificate delivered to Collateral Agent in connection herewith with respect of which Borrower has given Collateral Agent notice and taken such actions as are necessary to give Collateral Agent a perfected security interest therein.

On the Effective Date, the Collateral (other than raw materials or unfinished products that may be in transit or located at third party manufacturing sites) is not in the possession of any third party bailee (such as a warehouse) except as disclosed in the Perfection Certificate. None of the components of the Collateral (other than raw materials or unfinished products that may be in transit or located at third party manufacturing sites) shall be maintained at locations other than as disclosed in the Perfection Certificate on the Effective Date or as permitted pursuant to Section 7.2. In the event that Borrower, after the Effective Date, intends to store or otherwise deliver any portion of the Collateral to a bailee (other than raw materials or unfinished products that may be in transit or located at third party manufacturing sites), then Borrower will first receive the written consent of Collateral Agent and such bailee

must execute and deliver a bailee agreement in form and substance satisfactory to Collateral Agent in its reasonable discretion.

All Inventory is in all material respects of good and marketable (although not necessarily approved for sale to the public as regulated by the FDA) quality, free from material defects.

Borrower is the sole owner of its Intellectual Property, except for licenses permitted by the terms of Section 7.1 hereof and those licenses described in the Perfection Certificate. Schedule 5.2 sets forth all patents and patent applications owned or exclusively licensed to Borrower and indicates which of such patents and patent applications are owned by Borrower and which are licensed by Borrower from third parties (the "Licensed IP"). The Licensed IP is not necessary for the conduct of Borrower's Ataluren (PTC124), PTC299 and GEMS research and development programs nor the manufacture, sale and marketing of products, if any, which may be developed from such programs. Each issued Patent owned by Borrower is, to the best of Borrower's knowledge, valid and enforceable and no part of the Intellectual Property has been judged invalid or unenforceable, in whole or in part, and to the best of Borrower's knowledge, no claim has been made that any part of the Intellectual Property violates the rights of any third party except to the extent such claim could not reasonably be expected to have a material adverse effect on Borrower's business. Except as noted on the Perfection Certificate, Borrower is not a party to, nor is bound by, any material license or other agreement with respect to which Borrower is a licensee that (a) prohibits or otherwise restricts Borrower from granting a security interest in Borrower's interest in such license or agreement or any other property, or

(b) for which a default under or termination of could interfere with Collateral Agent's right to sell any Collateral. Borrower shall provide written notice to Collateral Agent within ten (10) days of entering or becoming bound by any such license or agreement (other than over-the-counter software that is commercially available to the public). In respect of such licenses or agreements, Borrower shall take such steps as Collateral Agent requests to obtain the consent of, or waiver by, any person whose consent or waiver is necessary for (x) all such licenses or agreements to be deemed "Collateral" and for Collateral Agent to have a security interest in it that might otherwise be restricted or prohibited by law or by the terms of any such license or agreement, whether now existing or entered into in the future, and (y) Collateral Agent to have the ability in the event of a liquidation of any Collateral to dispose of such Collateral in accordance with Collateral Agent's rights and remedies under this Agreement and the other Loan Documents. Notwithstanding the foregoing, the terms of the preceding sentence shall not apply to exclusive and non-exclusive license agreements solely for the use of the intellectual property of a third party in which Borrower is licensee.

5.3 Litigation. There are no actions or proceedings pending or, to the knowledge of the Responsible Officers, threatened in writing by or against Borrower or any of its Subsidiaries involving more than Two Hundred Fifty Thousand Dollars ($250,000.00).

5.4 No Material Deterioration in Financial Condition; Financial Statements. All consolidated financial statements for Borrower and any of its Subsidiaries delivered to Collateral Agent fairly present, in all material respects Borrower's consolidated financial condition and Borrower's consolidated results of operations on the dates of such statements or for the periods then ended. There has not been any material deterioration in Borrower's consolidated financial condition since the date of the most recent financial statements submitted to Collateral Agent.

5.5 Solvency. As of the Effective Date, the fair salable value of Borrower's assets (including Borrower's enterprise value minus disposition costs) exceeds the fair value of its liabilities; and Borrower is able to pay its debts (including trade debts) as they mature; provided, that for purposes of this Section 5.5, Borrower's liabilities shall not include liabilities attributable to deferred revenue (notwithstanding that recognition of such liabilities may be required by GAAP).

5.6 Regulatory Compliance. Borrower is not an "investment company" or a company "controlled" by an "investment company" under the Investment Company Act of 1940, as amended. Borrower is not engaged as one of its important activities in extending credit for margin stock (under Regulations X, T and U of the Federal Reserve Board of Governors). Borrower has complied in all material respects with the Federal Fair Labor Standards Act, if and as applicable to Borrower. Neither Borrower nor any of its Subsidiaries is a "holding company" or an "affiliate" of a "holding company" or a "subsidiary company" of a "holding company" as each term is defined and used in the Public Utility Holding Company Act of 2005. Borrower has not violated any laws, ordinances or rules, the violation of which could reasonably be expected to have a material adverse effect on its business. None of Borrower's or any of its Subsidiaries' properties or assets has been used by Borrower or any Subsidiary or, to Borrower's knowledge, by previous Persons, in disposing, producing, storing,

treating, or transporting any hazardous substance other than in material compliance with applicable laws. Borrower and each of its Subsidiaries have obtained all consents, approvals and authorizations of, made all declarations or filings with, and given all notices to, all Governmental Authorities that are necessary to continue their respective businesses as currently conducted.

None of the Borrower, its Affiliates or any of their respective agents acting or benefiting in any capacity in connection with the transactions contemplated by this Agreement is (i) in violation of any Anti-Terrorism Law, (ii) engages in or conspires to engage in any transaction that evades or avoids, or has the purpose of evading or avoiding or attempts to violate, any of the prohibitions set forth in any Anti-Terrorism Law, or (iii) is a Blocked Person. Neither Borrower nor, to the knowledge of Borrower, any of its Affiliates or agents acting or benefiting in any capacity in connection with the transactions contemplated by this Agreement, (x) conducts any business or engages in making or receiving any contribution of funds, goods or services to or for the benefit of any Blocked Person, or (y) deals in, or otherwise engages in any transaction relating to, any property or interest in property blocked pursuant to Executive Order No. 13224, any similar executive order or other Anti-Terrorism Law.

5.7 Subsidiaries; Investments. Borrower does not own any stock, partnership interest or other equity securities except for Permitted Investments.

5.8 Tax Returns and Payments; Pension Contributions. Borrower has timely filed all required tax returns and reports, and Borrower and its Subsidiaries have timely paid all foreign, federal, state and local taxes, assessments, deposits and contributions owed by Borrower, except for non-material tax reporting or payment deficiencies occurring prior to the Effective Date which have been cured. Borrower may defer payment of any contested taxes, provided that Borrower (a) in good faith contests its obligation to pay the taxes by appropriate proceedings promptly and diligently instituted and conducted, (b) notifies Collateral Agent in writing of the commencement of, and any material development in, the proceedings, and (c) posts bonds or takes any other steps required to prevent the governmental authority levying such contested taxes from obtaining a Lien upon any of the Collateral that is other than a "Permitted Lien". Borrower is unaware of any claims or adjustments proposed for any of Borrower's prior tax years which could result in additional taxes becoming due and payable by Borrower. Borrower has paid all amounts necessary to fund all present pension, profit sharing and deferred compensation plans in accordance with their terms, and Borrower has not withdrawn from participation in, and has not permitted partial or complete termination of, or permitted the occurrence of any other event with respect to, any such plan which could reasonably be expected to result in any liability of Borrower, including any liability to the Pension Benefit Guaranty Corporation or its successors or any other governmental agency.

5.9 Use of Proceeds. Borrower shall use the proceeds of the Credit Extensions solely as working capital and to fund its general business requirements and not for personal, family, household or agricultural purposes.

5.10 Full Disclosure. No written representation, warranty or other statement of Borrower in any certificate or written statement given to Collateral Agent or any Lender, as of the date such representation, warranty, or other statement was made, taken together with all such written certificates and written statements given to Collateral Agent or any Lender, contains any untrue statement of a material fact or omits to state a material fact necessary to make the statements contained in the certificates or statements not misleading (it being recognized that the projections and forecasts provided by Borrower in good faith and based upon reasonable assumptions are not viewed as facts and that actual results during the period or periods covered by such projections and forecasts may differ from the projected or forecasted results).

6 AFFIRMATIVE COVENANTS

Borrower shall do all of the following:

6.1 Government Compliance.

(a) Maintain its and all its Subsidiaries' legal existence and good standing in their respective jurisdictions of formation and maintain qualification in each jurisdiction in which the failure to so qualify could reasonably be expected to have a material adverse effect on Borrower's

business or operations. Borrower shall comply, and have each Subsidiary comply, with all laws, ordinances and regulations to which it is subject, the noncompliance with which could reasonably be expected to have a material adverse effect on Borrower's business.

(b) Obtain and keep in full force and effect, all of the Governmental Approvals necessary for the performance by Borrower of its obligations under the Loan Documents to which it is a party and the grant of a security interest to Collateral Agent for the ratable benefit of the Lenders, in all of the Collateral. Borrower shall promptly provide copies of any such obtained Governmental Approvals to Collateral Agent.

6.2 Financial Statements, Reports, Certificates.

(a) Deliver to Collateral Agent: (i) as soon as available, but no later than forty five (45) days after the last day of each fiscal quarter, a company prepared consolidated balance sheet and income statement covering Borrower's consolidated operations for such quarter certified by a Responsible Officer and in a form reasonably acceptable to Collateral Agent; (ii) no later than ten (10) Business Days after the last day of each month, a company prepared cash report certified by a Responsible Officer showing Borrower's cash balances as of the end of such month; (iii) as soon as available, but no later than thirty (30) days after the last day of each month, copies of the bank statements for each bank account maintained by Borrower; (iv) as soon as available, but no later than one hundred twenty (120) days after the last day of Borrower's fiscal year (commencing with Borrower's fiscal year ended December 31, 2009), audited consolidated financial statements prepared under GAAP, consistently applied, together with an unqualified opinion on the financial statements from an independent certified public accounting firm acceptable to Collateral Agent in its reasonable discretion (which shall include KPMG, the accounting firm utilized by the Borrower on the Effective Date); (v) as soon as available after approval thereof by Borrower's Board of Directors, Borrower's operating and capital budgets as approved by Borrower's Board of Directors; (vi) within five (5) days of delivery, copies of all statements, reports and notices made available to all of Borrower's security holders or to any holders of Subordinated Debt; (vii) in the event that Borrower becomes subject to the reporting requirements under the Securities Exchange Act of 1934, as amended, within five (5) days of filing, all reports on Form 10-K, 10-Q and 8-K filed with the Securities and Exchange Commission or a link thereto on Borrower's or another website on the Internet; (vi) a prompt report of any legal actions pending or threatened against Borrower or any of its Subsidiaries that could result in damages or costs to Borrower or any of its Subsidiaries of Two Hundred Fifty Thousand Dollars ($250,000.00) or more; and (viii) other financial information reasonably requested by Collateral Agent.

(b) Within thirty (30) days after the last day of each month, deliver to Collateral Agent, a duly completed Compliance Certificate signed by a Responsible Officer.

6.3 Inventory; Returns. Borrower and/or its agents shall keep all Inventory in good and marketable (although not necessarily approved for sale to the public as regulated by the FDA) condition, free from material defects and maintain such Inventory in accordance with appropriate storage requirements. Returns and allowances between Borrower (and/or Borrower's agents) and its Account Debtors shall follow Borrower's customary practices as they exist at the Effective Date or as are customary in the pharmaceutical industry. Borrower must promptly notify Collateral Agent of all returns, recoveries, disputes and claims that involve more than Two Hundred Fifty Thousand Dollars ($250,000).

6.4 Taxes; Pensions. Timely file and require each of its Subsidiaries to timely file, all required tax returns and reports and timely pay, and require each of its Subsidiaries to timely file and pay, all foreign, federal, state, and local taxes, assessments, deposits and contributions owed by Borrower and each of its Subsidiaries, except for deferred payment of any taxes contested pursuant to the terms of Section 5.8 hereof, and shall deliver to Collateral Agent, on demand, appropriate certificates attesting to such payments, and pay all amounts necessary to fund all present pension, profit sharing and deferred compensation plans in accordance with their terms.

6.5 Insurance. Keep its business and the Collateral insured for risks and in amounts standard for companies in Borrower's industry and location and as Collateral Agent may reasonably request. Insurance policies shall be in a form, with companies, and in amounts that are reasonably satisfactory to Collateral Agent, *provided however*, Collateral Agent acknowledges and agrees that Borrower's insurance companies and coverage in effect as of the Effective Date are satisfactory for Borrower's current properties and business. All property policies shall have a lender's loss payable endorsement showing Collateral Agent as lender loss payee and waive subrogation against Collateral Agent, and all liability policies shall show, or have endorsements showing, Collateral Agent, as an additional insured. All policies (or the loss payable and additional insured endorsements) shall provide that the insurer shall endeavor to give Collateral Agent at least thirty (30) days (or ten (10) days if due to non-payment of premium) notice before canceling, amending, or declining to renew its policy. At Collateral Agent's request, Borrower shall deliver certified copies of policies and evidence of all

premium payments. Proceeds payable under any casualty or business interruption policy shall, at Collateral Agent's option, be payable to Collateral Agent on behalf of the Lenders on account of the Obligations. Notwithstanding the foregoing, so long as no Event of Default has occurred and is continuing, (i) Borrower shall have the option of applying the proceeds of any casualty policy up to Two Hundred Fifty Thousand Dollars ($250,000) in the aggregate for all losses under all casualty policies in any one year, toward the replacement or repair of destroyed or damaged property, provided that any such replaced or repaired property (a) shall be of equal or like value as the replaced or repaired Collateral and (b) shall be deemed Collateral in which Collateral Agent has been granted a first priority security interest, and (ii) Borrower shall have the option of applying the proceeds of any casualty policy in excess of Two Hundred Fifty Thousand Dollars ($250,000) in the aggregate for all losses under all casualty policies in any one year, toward the replacement or repair of destroyed or damaged property, provided that (a) prior to the receipt and application of such proceeds, Borrower shall have delivered to the Collateral Agent and the Lenders a reinvestment plan detailing such replacement or repair which plan shall be acceptable to the Required Lenders in their reasonable discretion, (b) such proceeds are deposited into an account which is subject to a Control Agreement in favor of the Collateral Agent, (c) such proceeds shall be applied to such replacement or repair within one hundred eighty (180) days after receipt thereof by the Borrower (with any remaining unspent proceeds after such one hundred eighty (180) day period being payable to Collateral Agent for application to the Obligations), (d) any such replaced or repaired property (1) shall be of equal or like value as the replaced or repaired Collateral and (2) shall be deemed Collateral in which Collateral Agent has been granted a first priority security interest. In the event Lenders do not in their reasonable discretion approve of such a reinvestment plan detailing the replacement or repair of destroyed or damaged property, the related proceeds payable under any casualty policy shall, at the option of Collateral Agent, be payable to Collateral Agent on account of the Obligations. After the occurrence and during the continuance of an Event of Default, all proceeds payable under any casualty policy shall, at the option of Collateral Agent, be payable to Collateral Agent on account of the Obligations. In the event that any proceeds payable under any casualty policy are to be applied on account of the Obligations, Borrower shall cooperate with Collateral Agent to cause such proceeds to be paid to Collateral Agent, including, without limitation, executing any endorsements of such proceeds in favor of Collateral Agent. Borrower fails to obtain insurance as required under this Section 6.5 or to pay any amount or furnish any required proof of payment to third persons and Collateral Agent, Collateral Agent may make all or part of such payment or obtain such insurance policies required in this Section 6.5, and take any action under the policies Collateral Agent deems prudent.

6.6 Operating Accounts.

(a) Maintain all of Borrower's Collateral Accounts, operating and investment accounts with Borrower's Account Banks, which accounts are subject to Control Agreements in favor of Collateral Agent.

(b) Provide Collateral Agent five (5) days prior written notice before establishing any Collateral Account at or with any bank or financial institution other than Borrower's Account Banks. In addition, for each Collateral Account that Borrower at any time maintains, Borrower shall cause the applicable bank or financial institution (other than Borrower's Account Banks) at or with which any Collateral Account is maintained to execute and deliver a Control Agreement or other appropriate instrument with respect to such Collateral Account to perfect Collateral Agent's Lien in such Collateral Account in accordance with the terms hereunder, which Control Agreement may not be terminated without prior written consent of Collateral Agent. The provisions of the previous sentence shall not apply to deposit accounts exclusively used for payroll, payroll taxes and other employee wage and benefit payments to or for the benefit of Borrower's employees and identified to Collateral Agent by Borrower as such.

6.7 Protection of Intellectual Property Rights. Borrower shall: (a) use commercially reasonable efforts to protect, defend and maintain the validity and enforceability of its Intellectual Property; (b) promptly advise Collateral Agent in writing of material infringements of its Intellectual Property of which Borrower has knowledge; and (c) not allow any Intellectual Property material to Borrower's business to be abandoned, forfeited or dedicated to the public without Collateral Agent's written consent.

6.8 Litigation Cooperation. From the date hereof and continuing through the termination of this Agreement, make available to Collateral Agent, without expense to Collateral Agent, Borrower and its officers, employees and agents and Borrower's Books, to the extent that Collateral Agent may deem them reasonably necessary to prosecute or defend any third-party suit or proceeding instituted by or against Collateral Agent with respect to any Collateral or relating to Borrower provided that with respect to claim, suit, counterclaim or proceeding brought by a third party (i.e., other than the Collateral Agent, the Lenders or Borrower), Borrower may withhold such portion of Borrower's Books as would be reasonably be

required to protect Borrower's attorney-client privilege in respect of such third party claim, suit, counterclaim or proceeding (unless Borrower and the Collateral Agent or Lenders are parties to a joint defense agreement in which case no such information may be withheld).

6.9 Notices of Litigation and Default. Borrower will give prompt written notice to Collateral Agent of any litigation or governmental proceedings pending or threatened (in writing) against Borrower which would reasonably be expected to have a material adverse effect with respect to Borrower's business. Without limiting or contradicting any other more specific provision of this Agreement, promptly (and in any event within three (3) Business Days) upon Borrower becoming aware of the existence of any Event of Default or event which, with the giving of notice or passage of time, or both, would constitute an Event of Default, Borrower shall give written notice to Collateral Agent of such occurrence, which such notice shall include a reasonably detailed description of such Event of Default or event which, with the giving of notice or passage of time, or both, would constitute an Event of Default.

6.10 Creation/Acquisition of Subsidiaries. In the event Borrower or any Subsidiary creates or acquires any Subsidiary, Borrower and such Subsidiary shall promptly notify Collateral Agent of the creation or acquisition of such new Subsidiary and take all such action as may be reasonably required by Collateral Agent to cause each such Subsidiary to become a co-Borrower hereunder or to guarantee the Obligations of Borrower under the Loan Documents and, in each case, grant a continuing pledge and security interest in and to the assets of such Subsidiary (substantially as described on **Exhibit A** hereto); and Borrower shall grant and pledge to Collateral Agent, for the ratable benefit of the Lenders, a perfected security interest in the stock, units or other evidence of ownership of each Subsidiary.

6.11 New Drug Application. In the event Borrower borrows any Term B Loan, Borrower shall file a New Drug Application (NDA) with the Food and Drug Administration in respect of Borrower's Ataluren program on or before December 31, 2010.

6.12 Further Assurances.

(a) Execute any further instruments and take further action as Collateral Agent reasonably requests to perfect or continue Collateral Agent's Lien in the Collateral or to effect the purposes of this Agreement.

(b) Deliver to Collateral Agent, within ten (10) days after the same are sent or received, copies of all material correspondence, reports, documents and other filings received from any Governmental Authority that could reasonably be expected to have a material adverse effect on any of the Governmental Approvals material to Borrower's business or otherwise material to the operations of Borrower or any of its Subsidiaries.

(c) Deliver to Collateral Agent, within ten (10) days after request therefor from Collateral Agent or the Lenders, copies of all material correspondence, reports, documents and other filings sent by Borrower to any Governmental Authority or a summary thereof.

7 NEGATIVE COVENANTS

Borrower shall not do any of the following without the prior written consent of the Required Lenders:

7.1 Dispositions. Convey, sell, lease, transfer, assign, or otherwise dispose of (collectively, "Transfer"), or permit any of its Subsidiaries to Transfer, all or any part of its business or property, except for Transfers (a) of Inventory in the ordinary course of business; (b) of worn-out or obsolete or replaced Equipment; and (c) in connection with Permitted Liens and Permitted Investments; (d) of non-exclusive licenses for the use of the property, of Borrower or its Subsidiaries in the ordinary course of business; or (e) exclusive licenses for the use of the property, of Borrower or its Subsidiaries in connection with joint ventures and corporate collaborations provided each such exclusive license is specifically approved by Borrower's Board of Directors and approved with the prior written consent of the Required Lenders (which with respect to any Lender other than Oxford or MidCap Financial, LLC, a Delaware limited liability company (and its affiliates, herein referred to collectively as "MidCap") shall be approved in such Lender's reasonable discretion (other than with respect to exclusive licenses of PTC124 or PTC299 in all or substantially all the worldwide geographic regions in which the Borrower

retains rights to such drug or any license which would result in a "deemed liquidation" as defined in Borrower's Certificate of Incorporation, in respect of which such approval shall be granted in such Lender's sole discretion)). Notwithstanding the foregoing or any other provision of this Agreement, Borrower may Transfer all or any part of its business or property if either (i) Borrower obtains the prior written consent of the Required Lenders or (ii) Borrower repays the Term Loans in full in accordance with 2.2(d) and pays to the Lenders all amounts required to be paid pursuant to such Section 2.2(d) in connection with such prepayment (including, without limitation, the Final Payment and the Prepayment Fee) in full prior to or simultaneously with the consummation of such Transfer.

7.2 Changes in Business, Management, Ownership, or Business Locations. (a) Engage in or permit any of its Subsidiaries to engage in any business other than the businesses currently engaged in by Borrower and such Subsidiary, as applicable, or reasonably related thereto; (b) liquidate, wind up or dissolve or elect or resolve to liquidate, wind up or dissolve; or (c) (i) permit any Key Person to cease to be employed by Borrower or actively involved in the senior management of Borrower (unless such change is approved by the Borrower's Board of Directors and a replacement for such Key Person approved by the Borrower's Board of Directors is engaged within one hundred twenty (120) days), or (ii) enter into any transaction or series of related transactions in which the stockholders of Borrower who were not stockholders immediately prior to the first such transaction own more than 50% of the voting stock of Borrower immediately after giving effect to such transaction or related series of such transactions (other than by the sale of Borrower's equity securities in a public offering or to venture capital investors so long as Borrower identifies to Collateral Agent the venture capital investors prior to the closing of the transaction). Borrower shall not, without at least thirty (30) days prior written notice to Collateral Agent: (1) add any new offices or business locations, including warehouses (unless such new offices or business locations contain less than Twenty Thousand Dollars ($20,000) in Borrower's assets or property), (2) change its jurisdiction of organization, (3) change its organizational structure or type, (4) change its legal name, or (5) change any organizational number (if any) assigned by its jurisdiction of organization.

7.3 Mergers or Acquisitions. Merge or consolidate, or permit any of its Subsidiaries to merge or consolidate, with any other Person, or acquire, or permit any of its Subsidiaries to acquire, all or substantially all of the capital stock or property of another Person. A Subsidiary may merge or consolidate into another Subsidiary (provided such surviving Subsidiary is a "co-Borrower" hereunder or has provided a secured guaranty hereunder) or into Borrower provided Borrower is the surviving legal entity, and as long as no Event of Default is occurring prior thereto or arises as a result therefrom. Notwithstanding the foregoing or any other provision of this Agreement, Borrower may merge, consolidate, be acquired or acquire all or substantially all of the capital stock or property of another Person if either (i) Borrower obtains the prior written consent of the Required Lenders or (ii) Borrower repays the Term Loans in full in accordance with 2.2(d) and pays to the Lenders all amounts required to be paid pursuant to such Section 2.2(d) in connection with such prepayment (including, without limitation, the Final Payment and the Prepayment Fee) in full prior to or simultaneously with the consummation of such merger, consolidation or acquisition.

7.4 Indebtedness. Create, incur, assume, or be liable for any Indebtedness, or permit any Subsidiary to do so, other than Permitted Indebtedness.

7.5 Encumbrance. Create, incur, allow, or suffer any Lien on any of its property, or assign or convey any right to receive income, including the sale of any Accounts, or permit any of its Subsidiaries to do so, except for Permitted Liens, or permit any Collateral not to be subject to the first priority security interest granted herein (except for Permitted Liens that may have priority permitted by the terms of this Agreement), or enter into any agreement, document, instrument or other arrangement (except with or in favor of Collateral Agent) with any Person which directly or indirectly prohibits or has the effect of prohibiting Borrower or any Subsidiary from assigning, mortgaging, pledging, granting a security interest in or upon, or encumbering any of Borrower's or any Subsidiary's Intellectual Property (or any exclusive or non-exclusive license agreement whereby Borrower is the licensee of any Intellectual Property (other than any non-assignment provisions contained in such exclusive or non-exclusive license agreements)), except as is otherwise permitted in Section 7.1 hereof and the definition of "Permitted Liens" herein.

7.6 Maintenance of Collateral Accounts. Maintain any Collateral Account except pursuant to the terms of Section 6.6(b) hereof.

7.7 Distributions; Investments. (a) Pay any dividends (other than dividends payable solely in common stock) or make any distribution or payment or redeem, retire or purchase any capital stock (other than repurchases pursuant to the

terms of employee stock purchase plans, employee restricted stock agreements or similar plans), or (b) directly or indirectly make any Investment other than Permitted Investments, or permit any of its Subsidiaries to do so.

7.8 Transactions with Affiliates. Directly or indirectly enter into or permit to exist any material transaction with any Affiliate of Borrower, except for transactions that are in the ordinary course of Borrower's business, upon fair and reasonable terms that are no less favorable to Borrower than would be obtained in an arm's length transaction with a non-affiliated Person.

7.9 Subordinated Debt. (a) Make or permit any payment on any Subordinated Debt, except under the terms of the subordination, intercreditor, or other similar agreement to which such Subordinated Debt is subject, or (b) amend any provision in any document relating to the Subordinated Debt which would increase the amount thereof or adversely affect the subordination thereof to Obligations owed to the Lenders.

7.10 Compliance. Become an "investment company" or a company controlled by an "investment company", under the Investment Company Act of 1940, as amended or undertake as one of its important activities extending credit to purchase or carry margin stock (as defined in Regulation U of the Board of Governors of the Federal Reserve System), or use the proceeds of any Credit Extension for that purpose; fail to meet the minimum funding requirements of ERISA, permit a Reportable Event or Prohibited Transaction, as defined in ERISA, to occur; fail to comply with the Federal Fair Labor Standards Act or violate any other law or regulation, if the violation could reasonably be expected to have a material adverse effect on Borrower's business, or permit any of its Subsidiaries to do so; withdraw or permit any Subsidiary to withdraw from participation in, permit partial or complete termination of, or permit the occurrence of any other event with respect to, any present pension, profit sharing and deferred compensation plan which could reasonably be expected to result in any liability of Borrower, including any liability to the Pension Benefit Guaranty Corporation or its successors or any other governmental agency.

7.11 Compliance with Anti-Terrorism Laws. Collateral Agent hereby notifies Borrower that pursuant to the requirements of Anti-Terrorism Laws, and Collateral Agent's policies and practices, Collateral Agent is required to obtain, verify and record certain information and documentation that identifies Borrower and its principals, which information includes the name and address of Borrower and its principals and such other information that will allow Collateral Agent to identify such party in accordance with Anti-Terrorism Laws. Borrower will not, nor will Borrower permit any Subsidiary or Affiliate to, directly or indirectly, knowingly enter into any documents, instruments, agreements or contracts with any Person listed on the OFAC Lists. Borrower shall immediately notify Collateral Agent if Borrower has knowledge that Borrower or any Subsidiary or Affiliate is listed on the OFAC Lists or (a) is convicted on, (b) pleads *nolo contendere* to, (c) is indicted on, or (d) is arraigned and held over on charges involving money laundering or predicate crimes to money laundering. Borrower will not, nor will Borrower permit any Subsidiary or Affiliate to, directly or indirectly, (i) conduct any business or engage in any transaction or dealing with any Blocked Person, including, without limitation, the making or receiving of any contribution of funds, goods or services to or for the benefit of any Blocked Person, (ii) deal in, or otherwise engage in any transaction relating to, any property or interests in property blocked pursuant to Executive Order No. 13224, any similar

executive order or other Anti-Terrorism Law, or (iii) engage in or conspire to engage in any transaction that evades or avoids, or has the purpose of evading or avoiding, or attempts to violate, any of the prohibitions set forth in Executive Order No. 13224 or other Anti-Terrorism Law.

8 EVENTS OF DEFAULT

Any one of the following shall constitute an event of default (an "**Event of Default**") under this Agreement:

8.1 Payment Default. Borrower fails to (a) make any payment of principal or interest on any Credit Extension on its due date (unless such failure is the result of the failure of the Lenders to debit the Designated Deposit Account for such payment on the date when due and on such date sufficient monies were on deposit in the Designated Deposit Account to make such payment, in which case a three (3) Business Day grace period shall apply), or (b) pay any other Obligations within three (3) Business Days after such Obligations are due and payable (which three (3) Business Day grace period shall not apply to payments due on the Maturity Date or the date of acceleration pursuant to Section 9.1 (a) hereof). During the cure period, the failure to cure the payment default is not an Event of Default (but no Credit Extension will be made during the cure period);

8.2 Covenant Default.

(a) Borrower fails or neglects to perform any obligation in Sections 6.2, 6.4, 6.5, 6.6, or 6.10 or violates any covenant in Section 7; or

(b) Borrower or any of its Subsidiaries fails or neglects to perform, keep, or observe any other term, provision, condition, covenant or agreement contained in this Agreement or any Loan Documents, and as to any default (other than those specified in this Section 8) under such other term, provision, condition, covenant or agreement that can be cured, has failed to cure the default within ten (10) days after the occurrence thereof; provided, however, that if the default cannot by its nature be cured within the ten (10) day period or cannot after diligent attempts by Borrower be cured within such ten (10) day period, and such default is likely to be cured within a reasonable time, then Borrower shall have an additional period (which shall not in any case exceed thirty (30) days) to attempt to cure such default, and within such reasonable time period the failure to cure the default shall not be deemed an Event of Default (but no Credit Extensions shall be made during such cure period). Grace periods provided under this Section shall not apply, among other things, to financial covenants or any other covenants set forth in subsection (a) above;

8.3 Material Adverse Change. A Material Adverse Change occurs;

8.4 Attachment; Levy; Restraint on Business.

(a) (i) The service of process seeking to attach, by trustee or similar process, any funds of Borrower or of any entity under control of Borrower (including a Subsidiary) on deposit with the Lenders or any Lender Affiliate, or (ii) a notice of lien, levy, or assessment is filed against any of Borrower's assets by any government agency, and the same under subclauses (i) and (ii) hereof are not, within ten (10) days after the occurrence thereof, discharged or stayed (whether through the posting of a bond or otherwise); provided, however, no Credit Extensions shall be made during any ten (10) day cure period; and

(b) (i) any material portion of Borrower's assets is attached, seized, levied on, or comes into possession of a trustee or receiver, or (ii) any court order enjoins, restrains, or prevents Borrower from conducting any part of its business;

8.5 Insolvency (a) Borrower is unable to pay its debts (including trade debts) as they become due or otherwise becomes insolvent; (b) Borrower begins an Insolvency Proceeding; or (c) an Insolvency Proceeding is begun against Borrower and not dismissed or stayed within forty-five (45) days (but no Credit Extensions shall be made while any of the conditions described in clause (a) exist and/or until any Insolvency Proceeding is dismissed);

8.6 Other Agreements. There is a default in any agreement to which Borrower is a party with a third party or parties resulting in a right by such third party or parties, whether or not exercised, to accelerate the maturity of any Indebtedness in an amount in excess of Two Hundred Fifty Thousand Dollars ($250,000) or that could have a material adverse effect on Borrower's business.

8.7 Judgments. One or more judgments, orders, or decrees for the payment of money in an amount, individually or in the aggregate, of at least Two Hundred Fifty Thousand Dollars ($250,000) (not covered by independent third-party insurance as to which liability has been accepted by such insurance carrier) shall be rendered against Borrower and shall remain unsatisfied, unvacated, or unstayed for a period of ten (10) days after the entry thereof (provided that no Credit Extensions will be made prior to the satisfaction, vacation, or stay of such judgment, order or decree);

8.8 Misrepresentations. Borrower or any Person acting for Borrower makes any representation, warranty, or other statement now or later in this Agreement, any Loan Document or in any writing delivered to Collateral Agent and/or the Lenders or to induce Collateral Agent and/or the Lenders to enter this Agreement or any Loan Document, and such representation, warranty, or other statement is incorrect in any material respect when made;

8.9 Subordinated Debt. A default or breach occurs under any agreement between Borrower and any creditor of Borrower that signed a subordination, intercreditor, or other similar agreement with Collateral Agent or the Lenders, or

any creditor that has signed such a subordination, intercreditor, or other similar agreement with Collateral Agent or the Lenders breaches any terms of such agreement; or

8.10 Governmental Approvals. Any Governmental Approval applicable to Borrower shall have been (a) revoked, rescinded, suspended, modified in an adverse manner or not renewed in the ordinary course for a full term or (b) subject to any decision by a Governmental Authority that designates a hearing with respect to any applications for renewal of any of such Governmental Approval or that could result in the Governmental Authority taking any of the actions described in clause (a) above, and such decision or such revocation, rescission, suspension, modification or non-renewal (i) has, or could reasonably be expected to have, a Material Adverse Change, or (ii) adversely affects the legal qualifications of Borrower or any of its Subsidiaries to hold such Governmental Approval in

any applicable jurisdiction and such revocation, rescission, suspension, modification or non-renewal could reasonably be expected to affect the status of or legal qualifications of Borrower or any of its Subsidiaries to hold any Governmental Approval in any other jurisdiction.

8.11 Lien Priority. Except as permitted by Collateral Agent, any Lien created hereunder or by any other Loan Document shall at any time fail to constitute a valid and perfected Lien on all of the Collateral purported to be secured thereby, subject to no prior or equal Lien, other than Permitted Liens.

9 RIGHTS AND REMEDIES

9.1 Rights and Remedies.

(a) Upon the occurrence and during the continuance of an Event of Default, Collateral Agent may, and at the written direction of any Lender shall, without notice or demand, do any or all of the following: (i) deliver notice of the Event of Default to Borrower, (ii) by notice to Borrower declare all Obligations immediately due and payable (but if an Event of Default described in Section 8.5 occurs all Obligations shall be immediately due and payable without any action by Collateral Agent or the Lenders) or (iii) by notice to Borrower suspend or terminate the obligations, if any, of the Lenders to advance money or extend credit for Borrower's benefit under this Agreement or under any other agreement between Borrower and Collateral Agent and/or the Lenders (but if an Event of Default described in Section 8.5 occurs all obligations, if any, of the Lenders to advance money or extend credit for Borrower's benefit under this Agreement or under any other agreement between Borrower and Collateral Agent and/or the Lenders shall be immediately terminated without any action by Collateral Agent or the Lenders).

(b) Without limiting the rights of the Collateral Agent and the Lenders set forth in Section 9.1 (a) above, upon the occurrence and during the continuance of an Event of Default Collateral Agent shall have the right, at the written direction of the Required Lenders, without notice or demand, to do any or all of the following:

(i) foreclose upon and/or sell or otherwise liquidate, the Collateral;

(ii) apply to the Obligations any (a) balances and deposits of Borrower that Collateral Agent or any Lender holds or controls, or (b) any amount held or controlled by Collateral Agent or any Lender owing to or for the credit or the account of Borrower; and/or

(iii) commence and prosecute an Insolvency Proceeding or consent to the Borrower commencing any Insolvency Proceeding.

(c) Without limiting the rights of the Collateral Agent and the Lenders set forth in Sections 9.1(a) and (b) above, upon the occurrence and during the continuance of an Event of Default Collateral Agent shall have the right, without notice or demand, to do any or all of the following:

(i) settle or adjust disputes and claims directly with Account Debtors for amounts on terms and in any order that Collateral Agent considers advisable, notify any Person owing Borrower money of Collateral Agent's security interest in such funds, and verify the amount of such account;

(ii) make any payments and do any acts it considers necessary or reasonable to protect the Collateral and/or its security interest in the Collateral. Borrower shall assemble the Collateral if Collateral

Agent requests and make it available as Collateral Agent designates. Collateral Agent may enter premises where the Collateral is located, take and maintain possession of any part of the Collateral, and pay, purchase, contest, or compromise any Lien which appears to be prior or superior to its security interest and pay all expenses incurred. Borrower grants Collateral Agent a license to enter and occupy any of its premises, without charge, to exercise any of Collateral Agent's rights or remedies;

(iv) ship, reclaim, recover, store, finish, maintain, repair, prepare for sale, and/or advertise for sale, the Collateral. Collateral Agent is hereby granted a non-exclusive, royalty-free license or other right to use, without charge, Borrower's labels, patents, copyrights, mask works, rights of use of any name, trade secrets, trade names, trademarks, service marks, and advertising matter, or any similar property as it pertains to the Collateral, in completing production of, advertising for sale, and selling any Collateral and, in connection with Collateral Agent's exercise of its rights under this Section 9.1, Borrower's rights under all licenses and all franchise agreements inure to Collateral Agent for the benefit of the Lenders;

(v) place a "hold" on any account maintained with Collateral Agent or the Lenders and/or deliver a notice of exclusive control, any entitlement order, or other directions or instructions pursuant to any Control Agreement or similar agreements providing control of any Collateral;

(vi) demand and receive possession of Borrower's Books (other than computer software that Borrower is expressly prohibited from transferring by the terms of the agreement by which Borrower obtained such software); and

(vii) Subject to clauses 9.1(a) and (b), exercise all rights and remedies available to Collateral Agent under the Loan Documents or at law or equity, including all remedies provided under the Code (including disposal of the Collateral pursuant to the terms thereof).

Notwithstanding any provision of this Section 9.1 to the contrary, upon the occurrence of any Event of Default, Collateral Agent shall have the right to exercise any and all remedies referenced in this Section 9.1 without the written consent of Required Lenders following the occurrence of an Exigent Circumstance. As used in the immediately preceding sentence, "Exigent Circumstance" means any event or circumstance that, in the reasonable judgment of Collateral Agent, imminently threatens the ability of Collateral Agent to realize upon all or any material portion of the Collateral, such as, without limitation, fraudulent removal, concealment, or abscondment thereof, destruction or material waste thereof, or failure of Borrower after reasonable demand to maintain or reinstate adequate casualty insurance coverage, or which, in the judgment of Collateral Agent, could result in a material diminution in value of the Collateral.

9.2 Power of Attorney. Borrower hereby irrevocably appoints Collateral Agent as its lawful attorney-in-fact, exercisable upon the occurrence and during the continuance of an Event of Default, to: (a) endorse Borrower's name on any checks or other forms of payment or security; (b) sign Borrower's name on any invoice or bill of lading for any Account or drafts against Account Debtors; (c) settle and adjust disputes and claims about the Accounts directly with Account Debtors, for amounts and on terms Collateral Agent determines reasonable; (d) make, settle, and adjust all claims under Borrower's insurance policies; (e) pay,

contest or settle any Lien, charge, encumbrance, security interest, and adverse claim in or to the Collateral, or any judgment based thereon, or otherwise take any action to terminate or discharge the same; and (f) transfer the Collateral into the name of Collateral Agent or a third party as the Code permits. Borrower hereby appoints Collateral Agent as its lawful attorney-in-fact to sign Borrower's name on any documents necessary to perfect or continue the perfection of Collateral Agent's security interest in the Collateral regardless of whether an Event of Default has occurred until all Obligations have been satisfied in full and Collateral Agent and the Lenders are under no further obligation to make Credit Extensions hereunder. Collateral Agent's foregoing appointment as Borrower's attorney in fact, and all of Collateral Agent's rights and powers, coupled with an interest, are irrevocable until all Obligations have been fully repaid and performed and Collateral Agent's and the Lenders' obligation to provide Credit Extensions terminates.

9.3 Protective Payments. If Borrower fails to obtain the insurance called for by Section 6.5 or fails to pay any premium thereon or fails to pay any other amount which Borrower is obligated to pay under this Agreement or any other Loan Document, Collateral Agent may obtain such insurance or make such payment, and all amounts so paid by Collateral Agent are Lenders' Expenses and immediately due and payable, bearing interest at the then highest applicable rate, and secured by the Collateral. Collateral Agent will make reasonable efforts to provide Borrower with notice of Collateral Agent

obtaining such insurance at the time it is obtained or within a reasonable time thereafter. No such payments by Collateral Agent are deemed an agreement to make similar payments in the future or Collateral Agent's waiver of any Event of Default.

9.4 Application of Payments and Proceeds. Notwithstanding anything to the contrary contained in this Agreement, upon the occurrence and during the continuance of an Event of Default, (a) Borrower irrevocably waives the right to direct the application of any and all payments at any time or times thereafter received by Collateral Agent from or on behalf of Borrower of all or any part of the Obligations, and, as between Borrower on the one hand and Collateral Agent and Lenders on the other, Collateral Agent shall have the continuing and exclusive right to apply and to reapply any and all payments received against the Obligations in such manner as Collateral Agent may deem advisable notwithstanding any previous application by Collateral Agent, and (b) the proceeds of any sale of, or other realization upon all or any part of the Collateral shall be applied: first, to the Lenders Expenses; second, to accrued and unpaid interest on the Obligations (including any interest which, but for the provisions of the United States Bankruptcy Code, would have accrued on such amounts); third, to the principal amount of the Obligations outstanding; and fourth, to any other indebtedness or obligations of Borrower owing to Collateral Agent or any Lender under the Loan Documents. Any balance remaining shall be delivered to Borrower or to whoever may be lawfully entitled to receive such balance or as a court of competent jurisdiction may direct. In carrying out the foregoing, (x) amounts received shall be applied in the numerical order provided until exhausted prior to the application to the next succeeding category, and (y) each of the Persons entitled to receive a payment in any particular category shall receive an amount equal to its pro rata share of amounts available to be applied pursuant thereto for such category. Any reference in this Agreement to an allocation between or sharing by the Lenders of any right, interest or obligation "ratably," "proportionally" or in similar terms shall refer to Pro Rata Share unless expressly provided otherwise. Collateral Agent, or if applicable, each Lender, shall promptly remit to the other Lenders such sums as may be necessary to ensure the ratable repayment of each Lender's portion of any Term Loan and the ratable distribution of interest, fees and reimbursements paid or made by Borrower. Notwithstanding the foregoing, (a) a Lender receiving a scheduled payment shall not be responsible for determining whether the other Lenders also received their scheduled payment on such date; provided, however, if it is later determined that a Lender received more than its ratable share of scheduled payments made on any date or dates, then such Lender shall remit to the Collateral Agent or other Lenders such sums as may be necessary to ensure the ratable payment of such scheduled payments, as instructed by Collateral Agent and (b) for so long as Oxford is Collateral Agent and the holder of the Indebtedness represented by the Oxford Equipment Financing, all payments received by Collateral Agent in respect of the Obligations (other than payments representing the proceeds of the Equipment financed pursuant to the Oxford Equipment Financing) shall be applied in the following order of priority: (i) first, to the Obligations then due and payable to the Lenders under the Loan Documents, and (ii) second, to the payment of the Indebtedness represented by the Oxford Equipment Financing. Any payment or distribution of any kind or character, whether in cash, properties or securities, shall be received by a Lender in excess of its ratable share, then the portion of such payment or distribution in excess of such Lender's ratable share shall be received by such Lender in trust for and shall be promptly paid over to the other Lender for application to the payments of amounts due on the other Lender's claims. To the extent any payment for the account of Borrower is required to be returned as a voidable transfer or otherwise, the Lenders shall contribute to one another as is necessary to ensure that such return of payment is on a pro rata basis. If any Lender shall obtain possession of any Collateral, it shall hold such Collateral for itself and as agent and bailee for the Collateral Agent and other Lenders for purposes of perfecting Collateral Agent's security interest therein. Notwithstanding anything to the contrary herein, any warrants issued to the Lenders by Borrower, the stock issuable thereunder, any equity securities purchased by Lenders, any amounts paid thereunder, any dividends, and any other rights in connection therewith shall not be subject to the terms and conditions of this Agreement. Nothing herein shall affect any Lender's rights under any such warrants, stock, or other equity securities to administer, manage, transfer, assign, or exercise such warrants, stock, or other equity securities for its own account.

9.5 Liability for Collateral. So long as Collateral Agent and the Lenders consult with Borrower and to the extent practicable comply with reasonable banking practices regarding the safekeeping of the Collateral in the possession or under the control of Collateral Agent and the Lenders, Collateral Agent and the Lenders shall not be liable or responsible for: (a) the safekeeping of the Collateral; (b) any loss or damage to the Collateral; (c) any diminution in the value of the Collateral; or (d) any act or default of any carrier, warehouseman, bailee, or other Person. Borrower bears all risk of loss, damage or destruction of the Collateral.

9.6 No Waiver; Remedies Cumulative. Collateral Agent's failure, at any time or times, to require strict performance by Borrower of any provision of this Agreement or any other Loan Document shall not waive, affect, or diminish any right of Collateral Agent thereafter to demand strict performance and compliance herewith or therewith. No waiver hereunder shall be effective unless signed by Collateral Agent and then is only effective for the specific instance and

purpose for which it is given. Collateral Agent's rights and remedies under this Agreement and the other Loan Documents are cumulative. Collateral Agent has all rights and remedies provided under the Code, by law, or in equity. Collateral Agent's exercise of one right or remedy is not an election, and Collateral Agent's waiver of any Event of Default is not a continuing waiver. Collateral Agent's delay in exercising any remedy is not a waiver, election, or acquiescence.

9.7 Demand Waiver. Borrower waives, to the fullest extent permitted by law, demand, notice of default or dishonor, notice of payment and nonpayment, notice of any default, nonpayment at maturity, release, compromise, settlement, extension, or renewal of accounts, documents, instruments, chattel paper, and guarantees held by Collateral Agent on which Borrower is liable.

10 NOTICES

All notices, consents, requests, approvals, demands, or other communication (collectively, "**Communication**") by any party to this Agreement or any other Loan Document must be in writing and shall be deemed to have been validly served, given, or delivered: (a) upon the earlier of

actual receipt and three (3) Business Days after deposit in the U.S. mail, first class, registered or certified mail return receipt requested, with proper postage prepaid; (b) upon transmission, when sent by-electronic mail (if an email address is specified herein) or facsimile transmission; (c) one (1) Business Day after deposit with a reputable overnight courier with all charges prepaid; or (d) when delivered, if hand-delivered by messenger, all of which shall be addressed to the party to be notified and sent to the address, or facsimile number, or indicated below (with a copy to any appropriate email address indicated below). Any of Collateral Agent, Lender or Borrower may change its mailing or electronic mail address or facsimile number by giving the other party written notice thereof in accordance with the terms of this Section 10.

If to Borrower:	PTC Therapeutics, Inc. 100 Corporate Court Middlesex Business Center South Plainfield, New Jersey 07080 Attn: Legal Department Telephone: 908-222-7000 Fax: 908-222-1128

with a copy to (which shall not constitute notice):

Faber Daeufer & Rosenberg PC,
950 Winter Street
Suite 4500
Waltham, MA 02451
Attn: Joseph L. Faber
Fax: (781)795-4747
Email: Joe.faber@fdrpc.com

If to Collateral Agent or Oxford:	Oxford Finance Corporation 133 North Fairfax Street Alexandria, Virginia 22314 Attention: General Counsel Fax: (703) 519-5225
with a copy to:	Riemer & Braunstein LLP Three Center Plaza Boston, Massachusetts 02108 Attn: David A. Ephraim, Esquire Fax: (617) 880-3456 Email: DEphraim@riemerlaw.com
If to MidCap:	Midcap Financial, LLC 7735 Old Georgetown Rd, Suite 400 Bethesda, MD 20814 Attn: Loan Servicing — PTC Transaction

11 CHOICE OF LAW, VENUE AND JURY TRIAL WAIVER

New York law governs the Loan Documents without regard to principles of conflicts of law. Borrower, Lenders and Collateral Agent each submit to the exclusive jurisdiction of the State and Federal courts in New York. NOTWITHSTANDING THE FOREGOING, COLLATERAL AGENT AND LENDERS SHALL HAVE THE RIGHT TO BRING ANY ACTION OR PROCEEDING AGAINST BORROWER OR ITS PROPERTY IN THE COURTS OF ANY OTHER JURISDICTION WHICH COLLATERAL AGENT AND LENDERS (IN ACCORDANCE WITH THE PROVISIONS OF SECTION 9.1) DEEM NECESSARY OR APPROPRIATE TO REALIZE ON THE COLLATERAL OR TO OTHERWISE ENFORCE COLLATERAL AGENT'S AND LENDERS' RIGHTS AGAINST BORROWER OR ITS PROPERTY. Borrower expressly submits and consents in advance to such jurisdiction in any action or suit commenced in any state or federal court located in New York City, and Borrower hereby waives any objection that it may have based upon lack of personal jurisdiction, improper venue, or forum non conveniens and hereby consents to the granting of such legal or equitable relief as is deemed appropriate by such court. Borrower hereby waives personal service of the summons, complaints, and other process issued in such action or suit and agrees that service of such summons, complaints, and other process may be made by registered or certified mail addressed to Borrower at the address set forth in Section 10 of this Agreement and that service so made shall be deemed completed upon the earlier to occur of Borrower's actual receipt thereof or three (3) days after deposit in the U.S. mails, proper postage prepaid.

TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, BORROWER, THE LENDERS AND COLLATERAL AGENT EACH WAIVE THEIR RIGHT TO A JURY TRIAL OF ANY CLAIM OR CAUSE OF ACTION ARISING OUT OF OR BASED UPON THIS AGREEMENT, THE LOAN DOCUMENTS OR ANY CONTEMPLATED TRANSACTION, INCLUDING CONTRACT, TORT, BREACH OF DUTY AND ALL OTHER CLAIMS. THIS WAIVER IS A MATERIAL INDUCEMENT FOR THE PARTIES TO ENTER INTO THIS AGREEMENT. EACH PARTY HAS REVIEWED THIS WAIVER WITH ITS COUNSEL.

12 GENERAL PROVISIONS

12.1 Successors and Assigns. This Agreement binds and is for the benefit of the successors and permitted assigns of each party. Borrower may not assign this Agreement or any rights or obligations under it without Collateral Agent's prior written consent (which may be granted or withheld in Collateral Agent's discretion, subject to Section 12.11). The Lenders have the right, without the consent of or notice to Borrower, to sell, transfer, assign, negotiate, or grant

participation in all or any part of, or any interest in, the Lenders' obligations, rights, and benefits under this Agreement and the other Loan Documents; provided, however that so long as no Event of Default has occurred and is continuing, any such sale, assignment, negotiation or grant of a participation by any Lender (other than a sale, assignment or participation to a Qualified Assignee (as defined below)) of its obligations, rights, and benefits under this Agreement and the other Loan Documents shall require the prior written consent of Collateral Agent. As used herein, "**Qualified Assignee**" means (a) any Lender and any Affiliate of any Lender and (b) any commercial bank, savings and loan association or savings bank or any other entity which is an "accredited investor" (as defined in Regulation D under the Securities Act) which extends credit or buys loans as one of its businesses, including insurance companies, mutual funds, lease financing companies and commercial finance companies, in each case, which has a rating of BBB or higher from S&P and a rating of Baa2 or higher from Moody's at the date that it becomes a Lender and in each case of clauses (a) and (b), which, through its applicable lending office, is capable of lending to Borrower without the imposition of any withholding or similar taxes; provided that no person proposed to become a Lender after the Effective Date and determined by Collateral Agent to be acting in the capacity of a vulture fund or distressed debt purchaser shall be a Qualified Assignee, and no person or Affiliate of such person proposed to become a Lender after the Effective Date and that holds any subordinated debt or stock issued by Borrower shall be a Qualified Assignee.

12.2 Indemnification. Borrower agrees to indemnify, defend and hold Collateral Agent and the Lenders and their respective directors, officers, employees, agents, attorneys, or any other Person affiliated with or representing Collateral Agent or the Lenders (each, an "**Indemnified Person**") harmless against: (a) all obligations, demands, claims, and liabilities (collectively, "**Claims**") asserted by any other party in connection with the transactions contemplated by the Loan Documents; and (b) all losses or Lenders' Expenses incurred, or paid by Indemnified Person from, following, or arising from transactions between Collateral Agent, and/or the Lenders and Borrower (including reasonable attorneys' fees and expenses), except for Claims and/or losses directly caused by such Indemnified Person's gross negligence or willful misconduct (collectively, the "**Indemnified Liabilities**"). Borrower hereby further indemnifies, defends and holds each Indemnified Person harmless from and against any and all liabilities, obligations, losses, damages, penalties, actions, judgments, suits, claims, costs, expenses and disbursements of any kind or nature whatsoever (including the fees and disbursements of counsel for such Indemnitee) in connection with any investigative, response, remedial, administrative or judicial matter or proceeding, whether or not such Indemnified Person shall be designated a party thereto and including any such proceeding initiated by or on behalf of Borrower, and the reasonable expenses of investigation by engineers, environmental consultants and similar technical personnel and any commission, fee or compensation claimed by any broker (other than any broker retained by Collateral Agent or Lenders) asserting any right to payment for the transactions contemplated hereby which may be imposed on, incurred by or asserted against such Indemnified Person as a result of or in connection with the transactions contemplated hereby and the use or intended use of the proceeds of the loan proceeds.

12.3 Time of Essence. Time is of the essence for the performance of all Obligations in this Agreement.

12.4 Severability of Provisions. Each provision of this Agreement is severable from every other provision in determining the enforceability of any provision.

12.5 Correction of Loan Documents. Collateral Agent and the Lenders may correct patent errors and fill in any blank dates in this Agreement and the other Loan Documents consistent with the agreement of the parties.

12.6 Integration. This Agreement and the Loan Documents represent the entire agreement about this subject matter and supersede prior negotiations or agreements. All prior agreements, understandings, representations, warranties, and negotiations between the parties about the subject matter of this Agreement and the Loan Documents merge into this Agreement and the Loan Documents.

12.7 Counterparts. This Agreement may be executed in any number of counterparts and by different parties on separate counterparts, each of which, when executed and delivered, is an original, and all taken together, constitute one Agreement.

12.8 Survival. All covenants, representations and warranties made in this Agreement continue in full force until this Agreement has terminated pursuant to its terms and all Obligations (other than inchoate indemnity obligations and any other obligations which, by their terms, are to survive the termination of this Agreement) have been satisfied. The obligation of Borrower in Section 12.2 to indemnify each Lender and Collateral Agent shall survive until the statute of limitations with respect to such claim or cause of action shall have run.

12.9 Confidentiality. In handling any confidential information of Borrower, the Lenders and Collateral Agent shall exercise the same degree of care that it exercises for its own proprietary information, but disclosure of information may be made: (a) to the Lenders' and Collateral Agent's Subsidiaries or Affiliates who are bound by terms of confidentiality no less restrictive than those contained herein; (b) to prospective transferees or purchasers of any interest in the Credit Extensions (provided, however, the Lenders and Collateral Agent shall obtain such prospective transferee's or purchaser's agreement to the terms of this provision, unless an Event of Default has occurred and is continuing, in which case, the Lenders and Collateral Agent shall use commercially reasonable efforts to obtain such prospective transferee's or purchaser's agreement to the terms of this provision); (c) as required by law, regulation, subpoena, or other order; (d) to regulators or as otherwise required in connection with an examination or audit; (e) as Collateral Agent considers appropriate in exercising remedies under the Loan Documents; and (f) to third party service providers of the Lenders and/or Collateral Agent so long as such service providers have executed a confidentiality agreement with the Lenders and Collateral Agent with terms no less restrictive than those contained herein. Confidential information does not include information that either: (i) is in the public domain or in the Lenders' and/or Collateral Agent's possession when disclosed to the Lenders and/or Collateral Agent, or becomes part of the public domain after disclosure to the Lenders and/or Collateral Agent; or (ii) is disclosed to the Lenders and/or Collateral Agent by a third party, if the Lenders and/or Collateral Agent does not know that the third party is prohibited from disclosing the information.

Without limiting the foregoing, Collateral Agent may use confidential information for any purpose, including, without limitation, for the development of client databases, reporting purposes, and market analysis, so long as, in connection with the development of client databases, reporting purposes, and market analysis, Collateral Agent does not disclose Borrower's identity or the identity of any person associated with Borrower or any patentable subject matter unless otherwise expressly permitted by this Agreement. The provisions of the immediately preceding sentence shall survive the termination of this Agreement.

12.10 Right of Set Off. Borrower hereby grants to Collateral Agent and to each Lender, a lien, security interest and right of set off as security for all Obligations to Collateral Agent and each Lender hereunder, whether now existing or hereafter arising upon and against all deposits, credits, collateral and property, now or hereafter in the possession, custody, safekeeping or control of Collateral Agent or the Lenders or any entity under the control of Collateral Agent or the Lenders (including a Collateral Agent affiliate) or in transit to any of them. At any time after the occurrence and during the continuance of an Event of Default, without demand or notice, Collateral Agent or the Lenders may set off the same or any part thereof and apply the same to any liability or obligation of Borrower even though unmatured and regardless of the adequacy of any other collateral securing the Obligations. ANY AND ALL RIGHTS TO REQUIRE COLLATERAL AGENT TO EXERCISE ITS RIGHTS OR REMEDIES WITH RESPECT TO ANY OTHER COLLATERAL WHICH SECURES THE OBLIGATIONS, PRIOR TO EXERCISING ITS RIGHT OF SETOFF WITH RESPECT TO SUCH DEPOSITS, CREDITS OR OTHER PROPERTY OF BORROWER ARE HEREBY KNOWINGLY, VOLUNTARILY AND IRREVOCABLY WAIVED.

12.11 Amendments.

(a) No amendment, modification, termination or waiver of any provision of this Agreement or any other Loan Document, no approval or consent thereunder, or any consent to any departure by Borrower therefrom, shall in any event be effective unless the same shall be in writing and signed by Borrower, Collateral Agent and the Lenders having (x) more than 60% of the Term Loan Commitments of all Lenders or (y) if such Term Loan Commitments have expired or been terminated, more than 60% of the aggregate outstanding principal amount of the Term Loans (the "**Required Lenders**"); provided, however, that so long as a party that is a Lender hereunder on the Effective Date does not assign any portion of its Term Loan Commitment or Term Loan (other than to an Affiliate of such Lender), the "Required Lenders" shall include such Lender (or such Affiliate of such Lender to which such Lender may assign its interest). Except as set forth in clause (b) below, all such amendments, modifications, terminations or waivers requiring the consent of the "Lenders" shall require the written consent of Required Lenders.

(b) No amendment, modification, termination or waiver of any provision of this Agreement or any other Loan Document shall, unless in writing and signed by Collateral Agent and each Lender directly affected thereby: (i) increase or decrease the Commitment of any Lender (which shall be deemed to affect all Lenders), (ii) reduce the principal of or rate of interest on any Obligation or the amount of any fees payable hereunder (other than waiving the imposition of the Default Rate), (iii) postpone the date fixed for or waive any

payment of principal of or interest on any Term Loan, or any fees or reimbursement obligation hereunder, (iv) release all or substantially all of the Collateral, or consent to a transfer of any material Intellectual Property, in each case, except as otherwise expressly permitted in the Loan Documents (which shall be deemed to affect ail Lenders), (v) subordinate the lien granted in favor of Collateral Agent securing the Obligations (which shall be deemed to affect all Lenders), (vi) release a Loan Party from, or consent to a Loan Party's assignment or delegation of, such Loan Party's obligations hereunder and under the other Loan Documents or any Guarantor from its guaranty of the Obligations (which shall be deemed to affect all Lenders) or (vii) amend, modify, terminate or waive Section 9.4, 12.10 or 12.11(a) or this Section 12.11(b).

(c) Notwithstanding any provision in this Section 12.11 to the contrary, no amendment, modification, termination or waiver affecting or modifying the rights or obligations of Collateral Agent hereunder shall be effective unless signed by Borrower, Collateral Agent and Required Lenders.

Any amendment, modification, supplement, termination, waiver or consent pursuant to this Section 12.11 shall apply equally to, and shall be binding upon, all the Lenders and Collateral Agent.

12.12 Publicity.

Borrower will not directly or indirectly publish, disclose or otherwise use in any public disclosure, advertising material, promotional material, press release or interview, any reference to the name, logo or any trademark of Collateral Agent or any Lender or any of their Affiliates or any reference to this Agreement or the financing evidenced hereby, in any case except (a) as required by applicable law, subpoena or judicial or similar order, in which case Borrower shall endeavor to give Collateral Agent prior written notice of such publication or other disclosure, (b) with the consent of the Collateral Agent and of each Lender named in such public disclosure or press release, or (c) any subsequent disclosure of any information previously disclosed in accordance with this Section 12.12.

Each Lender and Borrower hereby authorizes each Lender to publish the name of such Lender and Borrower, the existence of the financing arrangements referenced under this Agreement, the primary purpose and/or structure of those arrangements, the amount of credit extended under each facility, the title and role of each party to this Agreement, and the total amount of the financing evidenced hereby in any "tombstone", comparable advertisement or press release which such Lender elects to submit for publication. In addition, each Lender and Borrower agrees that each Lender may provide lending industry trade organizations with information necessary and customary for inclusion in league table measurements after the Effective Date. With respect to any of the foregoing, such authorization shall be subject to such Lender providing Borrower and the other Lenders with an opportunity to review and confer with such Lender regarding, and approve, the contents of any such tombstone, advertisement or information, as applicable, prior to its initial submission for publication, but subsequent publications of the same tombstone, advertisement or information shall not require Borrower's approval.

13 COLLATERAL AGENT

13.1 Appointment and Authorization of Collateral Agent. Each Lender hereby irrevocably appoints, designates and authorizes Collateral Agent to take such action on its behalf under the provisions of this Agreement and each other Loan Document and to exercise such powers and perform such duties as are expressly delegated to it by the terms of this Agreement or any other Loan Document, together with such powers as are reasonably incidental thereto. Notwithstanding any provision to the contrary contained elsewhere herein or in any other Loan Document, Collateral Agent shall not have any duties or responsibilities, except those expressly set forth herein, nor shall Collateral Agent have or be deemed to have any fiduciary relationship with any Lender or participant, and no implied covenants, functions, responsibilities, duties, obligations or liabilities shall be read into this Agreement or any other Loan Document or otherwise exist against Collateral Agent. Without limiting the generality of the foregoing sentence, the use of the term "agent" herein and in the other Loan Documents with reference to Collateral Agent is not intended to connote any fiduciary or other implied (or express) obligations arising under agency doctrine of any applicable law. Instead, such term is used merely as a matter of market custom, and is intended to create or reflect only an administrative relationship between independent contracting parties.

13.2 Delegation of Duties. Collateral Agent may execute any of its duties under this Agreement or any other Loan Document by or through its, or its Affiliates', agents, employees or attorneys-in-fact and shall be entitled to obtain and rely upon the advice of counsel and other consultants or experts concerning all matters pertaining to such duties. Collateral Agent shall not be responsible for the negligence or misconduct of any agent or attorney-in-fact that it selects in the absence of gross negligence or willful misconduct.

13.3 Liability of Collateral Agent. Except as otherwise provided herein, no Collateral Agent-Related Person shall (a) be liable for any action taken or omitted to be taken by any of them under or in connection with this Agreement or any other Loan Document or the transactions contemplated hereby (except for its own gross negligence or willful misconduct in connection with its duties expressly set forth herein), or (b) be

responsible in any manner to any Lender or participant for any recital, statement, representation or warranty made by Borrower or any officer thereof, contained herein or in any other Loan Document, or in any certificate, report, statement or other document referred to or provided for in, or received by Collateral Agent under or in connection with, this Agreement or any other Loan Document, or the validity, effectiveness, genuineness, enforceability or sufficiency of this Agreement or any other Loan Document, or for any failure of Borrower or any other party to any Loan Document to perform its obligations hereunder or thereunder. No Collateral Agent-Related Person shall be under any obligation to any Lender or participant to ascertain or to inquire as to the observance or performance of any of the agreements contained in, or conditions of, this Agreement or any other Loan Document, or to inspect the properties, books or records of Borrower or any Affiliate thereof.

13.4 **Reliance by Collateral Agent**. Collateral Agent shall be entitled to rely, and shall be fully protected in relying, upon any writing, communication, signature, resolution, representation, notice, consent, certificate, affidavit, letter, telegram, facsimile, telex or telephone message, electronic mail message, statement or other document or conversation believed by it to be genuine and correct and to have been signed, sent or made by the proper Person or Persons, and upon advice and statements of legal counsel (including counsel to Borrower), independent accountants and other experts selected by Collateral Agent. Collateral Agent shall be fully justified in failing or refusing to take any action under any Loan Document unless it shall first receive such advice or concurrence of all Lenders as it deems appropriate and, if it so requests, it shall first be indemnified to its satisfaction by the Lenders against any and all liability and expense which may be incurred by it by reason of taking or continuing to take any such action. Collateral Agent shall in all cases be fully protected in acting, or in refraining from acting, under this Agreement or any other Loan Document in accordance with a request or consent of all Lenders and such request and any action taken or failure to act pursuant thereto shall be binding upon all the Lenders.

13.5 **Notice of Default**. Collateral Agent shall not be deemed to have knowledge or notice of the occurrence of any default and/or Event of Default, unless Collateral Agent shall have received written notice from a Lender or Borrower, describing such default or Event of Default. Collateral Agent will notify the Lenders of its receipt of any such notice. Collateral Agent shall take such action with respect to an Event of Default as may be directed in writing by the Required Lenders in accordance, with Article 9(a); provided, however, that while an Event of Default has occurred and is continuing, Collateral Agent may (but shall not be obligated to) take such action, or refrain from taking such action, with respect to such Event of Default as Collateral Agent shall deem advisable or in the best interest of the Lenders, including without limitation, satisfaction of other security interests, liens or encumbrances on the Collateral not permitted under the Loan Documents, payment of taxes on behalf of Borrower, payments to landlords, warehouseman, bailees and other persons in possession of the Collateral and other actions to protect and safeguard the Collateral, and actions with respect to insurance claims for casualty events affecting Borrower and/or the Collateral.

13.6 **Credit Decision; Disclosure of Information by Collateral Agent**. Each Lender acknowledges that no Collateral Agent-Related Person has made any representation or warranty to it, and that no act by Collateral Agent hereafter taken, including any consent to and acceptance of any assignment or review of the affairs of Borrower or any Affiliate thereof, shall be deemed to constitute any representation or warranty by any Collateral Agent-Related Person to any Lender as to any matter, including whether Collateral Agent-Related Persons have disclosed material information in their possession. Each Lender represents to Collateral Agent that it has, independently and without reliance upon any Collateral Agent-Related Person and based on such documents and information as it has deemed appropriate, made its own appraisal of, and investigation into, the business, prospects, operations, property, financial and other condition and creditworthiness of Borrower and its respective Subsidiaries,

and all applicable bank or other regulatory laws relating to the transactions contemplated hereby, and made its own decision to enter into this Agreement and to extend credit to Borrower hereunder. Each Lender also represents that it will, independently and without reliance upon any Collateral Agent-Related Person and based on such documents and information as it shall deem appropriate at the time, continue to make its own credit analysis, appraisals and decisions in taking or not taking action under this Agreement and the other Loan Documents, and to make such investigations as it deems necessary to inform itself as to the business, prospects, operations, property, financial and other condition and creditworthiness of Borrower. Except for notices, reports and other documents expressly required to be furnished to the Lenders by Collateral Agent herein, Collateral Agent shall not have any duty or responsibility to provide any Lender with any credit or other information concerning the business, prospects, operations, property, financial and other condition or creditworthiness of Borrower or any of its Affiliates which may come into the possession of any Collateral Agent-Related Person.

13.7 **Indemnification of Collateral Agent**. Whether or not the transactions contemplated hereby are consummated, each Lender shall, severally and pro rata based on its respective Pro Rata Share, indemnify upon demand each Collateral Agent-Related Person (to the extent not reimbursed by or on behalf of Borrower and without limiting the obligation of Borrower to do so), and hold harmless each Collateral Agent-Related Person from and against any and all Indemnified Liabilities (which shall not include legal expenses of Collateral Agent incurred in connection with the closing of the transactions contemplated by this Agreement) incurred by it; provided, however, that no Lender shall be liable for the payment to any Collateral Agent-Related Person of any portion of such Indemnified Liabilities to the extent determined in a judgment by a court of competent jurisdiction to have resulted from such Collateral Agent-Related Person's own gross negligence or willful misconduct; provided, however, that no action taken in accordance with the directions of the Required Lenders shall be deemed to constitute gross negligence or willful misconduct for purposes of this Section 13.7. Without limitation of the foregoing, each Lender shall, severally and pro rata based on its respective Pro Rata Share, reimburse Collateral Agent upon demand for its ratable share of any costs or out-of-pocket expenses (including Lenders' Expenses incurred after the closing of the transactions contemplated by this Agreement) incurred by Collateral Agent (in its capacity as Collateral Agent, and not as a Lender) in connection with the preparation, execution, delivery, administration, modification, amendment or enforcement (whether through negotiations, legal proceedings or otherwise) of, or legal advice in respect of rights or responsibilities under, this Agreement, any other Loan Document, or any document contemplated by or referred to herein, to the extent that Collateral Agent is not reimbursed for such expenses by or on behalf of Borrower. The undertaking in this Section 13.7 shall survive the payment in full of the Obligations, the termination of this Agreement and the resignation of Collateral Agent.

13.8 **Collateral Agent in its Individual Capacity**. With respect to its Credit Extensions, Oxford shall have the same rights and powers under this Agreement as any other Lender and may exercise such rights and powers as though it were not Collateral Agent, and the terms "Lender" and "Lenders" include Oxford in its individual capacity.

13.9 **Successor Collateral Agent**. Collateral Agent may resign as Collateral Agent upon ten (10) days' notice to the Lenders. If Collateral Agent resigns under this Agreement, all Lenders shall appoint from among the Lenders (or the affiliates thereof) a successor Collateral Agent for the Lenders, which successor Collateral Agent shall (unless an Event of Default has occurred and is continuing) be subject to the approval of Borrower (which approval shall not be unreasonably withheld or delayed). If no successor Collateral Agent is appointed prior to the effective date of the resignation of Collateral Agent, Collateral Agent may appoint, after consulting with the Lenders, a successor Collateral Agent from among the Lenders (or the affiliates thereof). Upon the acceptance of its appointment as successor Collateral Agent hereunder, the Person acting as such successor Collateral Agent shall succeed to all the rights, powers and duties of the retiring Collateral Agent and the respective term "Collateral Agent" means such successor Collateral Agent and the retiring Collateral Agent's appointment, powers and duties in such capacities shall be terminated without any other further act or deed on its behalf. After any retiring Collateral Agent's resignation hereunder as Collateral Agent, the provisions of this Article 13 and Sections

2.4(d) and 12.2 shall inure to its benefit as to any actions taken or omitted to be taken by it while it was Collateral Agent under this Agreement. If no successor Collateral Agent has accepted appointment as Collateral Agent by the date ten (10) days following a retiring Agent's notice of resignation, the retiring Agent's resignation shall nevertheless thereupon become effective and the Lenders shall perform all of the duties of Collateral Agent hereunder until such time, if any, as the Lenders appoint a successor agent as provided for above.

13.10 **Collateral Agent May File Proofs of Claim**. In case of the pendency of any receivership, insolvency, liquidation, bankruptcy, reorganization, arrangement, adjustment, composition or other judicial proceeding relative to Borrower, Collateral Agent (irrespective of whether the principal of any Loan, shall then be due and payable as herein expressed or by declaration or otherwise and irrespective of whether Collateral Agent shall have made any demand on Borrower) shall be entitled and empowered, by intervention in such proceeding or otherwise:

(a) to file and prove a claim for the whole amount of the principal and interest owing and unpaid in respect of the Credit Extensions and all other Obligations that are owing and unpaid and to file such other documents as may be necessary or advisable in order to have the claims of the Lenders and Collateral Agent (including any claim for the reasonable compensation, expenses, disbursements and advances of the Lenders and Collateral Agent and their respective agents and counsel and all other amounts due the Lenders and Collateral Agent allowed in such judicial proceeding); and

(b) to collect and receive any monies or other property payable or deliverable on any such claims and to distribute the same;

and any custodian, receiver, assignee, trustee, liquidator, sequestrator or other similar official in any such judicial proceeding is hereby authorized by each Lender to make such payments to Collateral Agent and, in the event that Collateral Agent shall consent to the making of such payments directly to the Lenders, to pay to Collateral Agent any amount due for the reasonable compensation, expenses, disbursements and advances of Collateral Agent and its agents and counsel, and any other amounts due Collateral Agent under Section 2.4(d). To the extent that Collateral Agent fails timely to do so, each Lender may file a claim relating to such Lender's claim.

13.11 **Collateral and Guaranty Matters**. The Lenders irrevocably authorize Collateral Agent, at its option and in its discretion, to release any Guarantor and any Lien on any Collateral granted to or held by Collateral Agent under any Loan Document (i) upon the date that all Obligations due hereunder have been fully and indefeasibly paid in full and no Term Loan Commitments or other obligations of any Lender to provide funds to Borrower under this Agreement remain outstanding, (ii) that is transferred or to be transferred as part of or in connection with any Transfer permitted hereunder or under any other Loan Document, or (iii) as approved in accordance with Section 12.11. Upon request by Collateral Agent at any time, all Lenders will confirm in writing Collateral Agent's authority to release its interest in particular types or items of Property, pursuant to this Section 13.11.

13.12 **Cooperation of Borrower**. If necessary, Borrower agrees to (i) execute any documents (including new Secured Promissory Notes) reasonably required to effectuate and acknowledge each assignment of a Term Loan Commitment or Loan to an assignee in accordance with Section 12.1, (ii) make Borrower's management reasonably available upon prior notice to meet with Collateral Agent and prospective participants and assignees of Term Loan Commitments or Credit Extensions and (iii) assist Collateral Agent or the Lenders in the preparation of information relating to the financial affairs of Borrower as any prospective participant or assignee of a Term Loan Commitment or Term Loan reasonably may request. Subject to the provisions of Section 12.9 Borrower authorizes each Lender to disclose to any prospective participant or assignee of a Term Loan Commitment, any and all information in such Lender's possession concerning Borrower and its financial affairs which has been delivered to such Lender by or on behalf of Borrower pursuant to this Agreement, or which has been delivered to such Lender by or on behalf of Borrower in connection with such Lender's credit evaluation of Borrower prior to entering into this Agreement.

14 DEFINITIONS

14.1 **Definitions**. As used in this Agreement, the following terms have the following meanings:

"**Account**" is any "account" as defined in the Code with such additions to such term as may hereafter be made, and includes, without limitation, all accounts receivable and other sums owing to Borrower.

"**Account Debtor**" is any "account debtor" as defined in the Code with such additions to such term as may hereafter be made.

"**Affiliate**" of any Person is a Person that owns or controls directly or indirectly the Person, any Person that controls or is controlled by or is under common control with the Person, and each of that Person's senior executive officers, directors, partners and, for any Person that is a limited liability company, that Person's managers and members.

"**Agreement**" is defined in the preamble hereof.

"**Amortization Date**" is, (i) with respect Term Loan A, March 1, 2010, and (b) with respect to Term Loan B, the 8th Payment Date following the Funding Date of Term Loan B.

"**Anti-Terrorism Laws**" means any Laws relating to terrorism or money laundering, including Executive Order No. 13224 (effective September 24, 2001), the USA PATRIOT Act, the Laws comprising or implementing the Bank Secrecy Act, and the Laws administered by OFAC.

"**Basic Rate**" is, with respect to a Term Loan, the per annum rate of interest (based on a year of 360 days) equal to the greater of (i) 13.65% and (ii) the sum of (a) the three-month U.S. LIBOR rate reported on Bankrate.com three (3) Business Days prior to the Funding Date of such Term Loan, plus (b) 12.40%.

"**Blocked Person**" means any Person: (a) listed in the annex to, or is otherwise subject to the provisions of, Executive Order No. 13224, (b) a Person owned or controlled by, or acting for or on behalf of, any Person that is listed in the annex to, or is otherwise subject to the provisions of, Executive Order No. 13224, (c) a Person with which any Lender is prohibited from dealing or otherwise engaging in any transaction by any Anti-

Terrorism Law, (d) a Person that commits, threatens or conspires to commit or supports "terrorism" as defined in Executive Order No. 13224, or (e) a Person that is named a "specially designated national" or "blocked person" on the most current list published by OFAC or other similar list.

"**Borrower**" is defined in the preamble hereof.

"**Borrower's Books**" are all Borrower's books and records including ledgers, federal and state tax returns, records regarding Borrower's assets or liabilities, the Collateral, business operations or financial condition, and all computer programs or storage or any equipment containing such information.

"**Borrowing Resolutions**" are, with respect to any Person, those resolutions adopted by such Person's Board of Directors and delivered by such Person to Collateral Agent approving the Loan Documents to which such Person is a party and the transactions contemplated thereby, together with a certificate executed by its secretary on behalf of such Person certifying that (a) such Person has the authority to execute, deliver, and perform its obligations under each of the Loan Documents to which it is a party, (b) that attached as Exhibit A to such certificate is a true, correct, and complete copy of the resolutions then in full force and effect authorizing and ratifying the execution, delivery, and performance by such Person of the Loan Documents to which it is a party, (c) the name(s) of the Person(s) authorized to execute the Loan Documents on behalf of such Person, together with a sample of the true signature(s) of such Person(s), and (d) that Collateral Agent and the Lenders may conclusively rely on such certificate unless and until such Person shall have delivered to Collateral Agent a further certificate canceling or amending such prior certificate.

"**Business Day**" is any day that is not a Saturday, Sunday or a day on which Collateral Agent is closed.

"**Cash Equivalents**" are (a) marketable direct obligations issued or unconditionally guaranteed by the United States or any agency or any State thereof having maturities of not more than one (1) year from the date of acquisition; (b) commercial paper maturing no more than one (1) year after its creation and having the highest rating from either Standard & Poor's Ratings Group or Moody's Investors Service, Inc., and (c) certificates of deposit issued maturing no more than one (1) year after issue. For the avoidance of doubt, the direct purchase by Borrower, co-borrower, or any subsidiary of Borrower of any Auction Rate Securities, or purchasing participations in, or

entering into any type of swap or other derivative transaction, or otherwise holding or engaging in any ownership interest in any type of Auction Rate Security by Borrower, co-borrower, or any subsidiary of Borrower shall be conclusively determined by the Lenders as an ineligible Cash Equivalent, and any such transaction shall expressly violate each other provision of this agreement governing Permitted Investments; *provided however*, that Borrower shall not be prohibited from engaging in transactions to hedge against currency fluctuations up to a maximum of $5,000,000 provided that such transactions are effected in accordance with a foreign exchange hedge policy that has been approved by Borrower's Board of Directors and the Collateral Agent; and *provided further*, that in the event that a foreign exchange hedge policy has been approved by Borrower's Board of Directors but not the Collateral Agent, Borrower shall not be prohibited from engaging in transactions to hedge against currency fluctuations up to a maximum of $500,000. Notwithstanding the foregoing, Cash Equivalents does not include and each Borrower and Subsidiary is prohibited from purchasing, purchasing participations in, entering into any type of swap or other equivalent derivative transaction, or otherwise holding or engaging in any ownership interest in any type of debt instrument, including, without limitation, any corporate or municipal bonds with a long-term nominal maturity for which the interest rate is reset through a dutch auction and more commonly referred to as an auction rate security; *provided however*, that Borrower shall not be prohibited from engaging in transactions to hedge against currency fluctuations up to a maximum of $5,000,000 provided that such transactions are effected in accordance with a foreign exchange hedge policy that has been approved by Borrower's Board of Directors and the Collateral Agent and; and *provided further*, that in the event that a foreign exchange hedge policy has been approved by Borrower's Board of Directors but not the Collateral Agent, Borrower shall not be prohibited from engaging in transactions to hedge against currency fluctuations up to a maximum of $500,000.

"**Claims**" are defined in Section 12.2.

"**Code**" is the Uniform Commercial Code, as the same may, from time to time, be enacted and in effect in the State of New York; provided, that, to the extent that the Code is used to define any term herein or in any Loan Document and such term is defined differently in different Articles or Divisions of the Code, the definition of such term contained in Article or Division 9 shall govern; provided further, that in the event that, by reason of mandatory provisions of law, any or all of the attachment, perfection, or priority of, or remedies with respect to, Collateral Agent's Lien on any Collateral is governed by the Uniform Commercial Code in effect in a jurisdiction other than the State of New York, the term "**Code**" shall mean the Uniform Commercial Code as enacted and in effect in such other jurisdiction solely for purposes of the provisions thereof relating to such attachment, perfection, priority, or remedies and for purposes of definitions relating to such provisions.

"**Collateral**" is any and all properties, rights and assets of Borrower described on **Exhibit A**.

"**Collateral Account**" is any Deposit Account, Securities Account, or Commodity Account.

"**Collateral Agent**" means, Oxford, not in its individual capacity, but solely in its capacity as agent on behalf of and for the benefit of the Lenders.

"**Collateral Agent-Related Person**" means the Collateral Agent, together with its Affiliates, and the officers, directors, employees, agents, advisors, auditors and attorneys-in-fact of such Persons; provided, however, that no Collateral Agent-Related Person shall be an Affiliate of Borrower.

"**Commitment Percentage**" is set forth in Schedule 1.1, as amended from time to time.

"**Commodity Account**" is any "commodity account" as defined in the Code with such additions to such term as may hereafter be made.

"**Communication**" is defined in Section 10.

"**Compliance Certificate**" is that certain certificate in the form attached hereto as **Exhibit C**.

"**Contingent Obligation**" is, for any Person, any direct or indirect liability, contingent or not, of that Person for (a) any indebtedness, lease, dividend, letter of credit or other obligation of another such as an obligation directly or indirectly guaranteed, endorsed, co-made, discounted or sold with recourse by that Person, or for which that Person is directly or indirectly liable; (b) any obligations for undrawn letters of credit for the account of that Person; and (c) all obligations from any interest rate, currency or commodity swap agreement, interest rate cap or collar agreement, or other agreement or arrangement designated to protect a Person against fluctuation in interest rates, currency exchange rates or commodity prices; but "Contingent Obligation" does not include endorsements in the ordinary course of business. The amount of a Contingent Obligation is the stated or determined amount of the primary obligation for which the Contingent Obligation is made or, if not determinable, the maximum reasonably anticipated liability for it determined by the Person in good faith; but the amount may not exceed the maximum of the obligations under any guarantee or other support arrangement.

"**Control Agreement**" is any control agreement entered into among the depository institution at which Borrower maintains a Deposit Account or the securities intermediary or commodity intermediary at which Borrower maintains a Securities Account or a Commodity Account, Borrower, and Collateral Agent pursuant to which Collateral Agent obtains control (within the meaning of the Code) for the benefit of the Lenders over such Deposit Account, Securities Account, or Commodity Account.

"**Credit Extension**" is any Term Loan or any other extension of credit by Collateral Agent or the Lenders for Borrower's benefit.

"**Default Rate**" is defined in Section 2.3(b).

"**Deposit Account**" is any "deposit account" as defined in the Code with such additions to such term as may hereafter be made.

"**Designated Deposit Account**" is Borrower's deposit account, account number 2000030197279, maintained with Wachovia Bank.

"**Dollars**" "**dollars**" and "**$**" each mean lawful money of the United States.

"**Effective Date**" is defined in the preamble of this Agreement.

"**Equipment**" is all "equipment" as defined in the Code with such additions to such term as may hereafter be made, and includes without limitation all machinery, fixtures, goods, vehicles (including motor vehicles and trailers), and any interest in any of the foregoing.

"**ERISA**" is the Employee Retirement Income Security Act of 1974, and its regulations.

"**Event of Default**" is defined in Section 8.

"**Final Payment**" is a payment (which payment is in addition to and not a substitution for the regular monthly payments of principal plus accrued interest and is intended as yield enhancement, not as a penalty) due on the earlier to occur of (a) the Maturity Date, or (b) the acceleration of any Term Loan, or (c) the prepayment of a Term Loan pursuant to Section 2.2(c) or (d), equal to the original principal amount of such Term Loan multiplied by the Final Payment Percentage, payable to the Lenders in accordance with their respective Pro Rata Shares.

"**Final Payment Percentage**" is three percent (3.00%).

"**First Draw Period**" is the period commencing on the Effective Date and ending on September 25, 2009.

"**Funding Date**" is any date on which a Credit Extension is made to or on account of Borrower which shall be a Business Day.

"**GAAP**" is generally accepted accounting principles set forth in the opinions and pronouncements of the Accounting Principles Board of the American Institute of Certified Public Accountants and statements and pronouncements of the Financial Accounting Standards Board or in such other statements by such other Person as may be approved by a significant segment of the accounting profession in the United States, which are applicable to the circumstances as of the date of determination.

"**General Intangibles**" is all "general intangibles" as defined in the Code in effect on the date hereof with such additions to such term as may hereafter be made, and includes without limitation, all copyright rights, copyright applications, copyright registrations and like protections in each work of authorship and derivative work, whether published or unpublished, any patents, trademarks, service marks and, to the extent permitted under applicable law, any applications therefor, whether registered or not, any trade secret rights, including any rights to unpatented inventions, payment intangibles, royalties, contract rights, goodwill, franchise agreements, purchase orders, customer lists, route lists, telephone numbers, domain names, claims, income and other tax refunds, security and other deposits, options to purchase or sell real or personal property, rights in all litigation presently or hereafter pending (whether in contract, tort or otherwise), insurance policies (including without limitation key man, property damage, and business interruption insurance), payments of insurance and rights to payment of any kind.

"**Governmental Approval**" is any consent, authorization, approval, order, license, franchise, permit, certificate, accreditation, registration, filing or notice, of, issued by, from or to, or other act by or in respect of, any Governmental Authority.

"**Governmental Authority**" is any nation or government, any state or other political subdivision thereof, any agency, authority, instrumentality, regulatory body, court, central bank or other entity exercising executive, legislative, judicial, taxing, regulatory or administrative functions of or pertaining to government, any securities exchange and any self-regulatory organization that has any authority or jurisdiction over Borrower and/or Borrower's business and operations.

"**Guarantor**" is any present or future guarantor of the Obligations.

"**Indebtedness**" is (a) indebtedness for borrowed money or the deferred price of property or services, such as reimbursement and other obligations for surety bonds and letters of credit, (b) obligations evidenced by notes, bonds, debentures or similar instruments, (c) capital lease obligations, and (d) Contingent Obligations.

"**Indemnified Liabilities**" is defined in Section 12.2.

"**Indemnified Person**" is defined in Section 12.2.

"**Insolvency Proceeding**" is any proceeding by or against any Person under the United States Bankruptcy Code, or any other bankruptcy or insolvency law, including assignments for the benefit of creditors, compositions, extensions generally with its creditors, or proceedings seeking reorganization, arrangement, or other relief.

"**Intellectual Property**" includes without limitation, all copyright rights, copyright applications, copyright registrations and like protections in each work of authorship and derivative work, whether published or unpublished, any patents, patent applications and like protections, including improvements, divisions, continuations, renewals, reissues, extensions, and continuations-in-part of the same, trademarks, trade names, service marks, mask works, rights of use of any name, domain names, or any other similar rights, any applications therefor, whether registered or not, and the goodwill of the business of Borrower connected with and symbolized thereby, know-how, operating manuals, trade secret rights, clinical and non-clinical data, rights to unpatented inventions, and any claims for damage by way of any past, present, or future infringement of any of the foregoing.

"**Inventory**" is all "inventory" as defined in the Code in effect on the date hereof with such additions to such term as may hereafter be made, and includes without limitation all merchandise, raw materials, parts, supplies, packing and shipping materials, work in process and finished products, including without limitation such inventory as is temporarily out of Borrower's custody or possession or in transit and including any returned goods and any documents of title representing any of the above.

"**Investment**" is any beneficial ownership interest in any Person (including stock, partnership interest or other securities), and any loan, advance or capital contribution to any Person.

"**Key Person**" is each of Borrower's President & Chief Executive Officer, Chief Medical Officer, Senior Vice President — Research and CMC, Senior Vice President — Commercial, and Chief Financial Officer, who are Stuart W. Peltz, Langdon Miller, Neil Almstead, Theresa Natalicchio, and William Baird, III, respectively, as of the Effective Date.

"**Lender**" is any one of the Lenders.

"**Lenders**" shall mean the Persons identified on Schedule 1.1 hereto and each assignee that becomes a party to this Agreement pursuant to Section 12.1.

"**Lenders' Expenses**" are all audit fees and expenses, costs, and expenses (including reasonable attorneys' fees and expenses) for preparing, amending, negotiating, administering, defending and enforcing the Loan Documents (including, without limitation, those incurred in connection with appeals or Insolvency Proceedings) or otherwise incurred by Collateral Agent or the Lenders in connection with the Loan Documents.

"**Lien**" is a claim, mortgage, deed of trust, levy, charge, pledge, security interest or other encumbrance of any kind, whether voluntarily incurred or arising by operation of law or otherwise against any property.

"**Loan Documents**" are, collectively, this Agreement, the Warrant, the Perfection Certificate, any note, or notes or guaranties executed by Borrower, and any other present or future agreement between Borrower and/or for the benefit of the Lenders and Collateral Agent in connection with this Agreement, all as amended, restated, or otherwise modified.

"**Loan Party**" is Borrower and each Guarantor.

"**Material Adverse Change**" is (a) a material impairment in the perfection or priority of the Collateral Agent's Lien in the Collateral or in the value of such Collateral; (b) a material adverse change in the business, operations, or condition (financial or otherwise) or prospects of Borrower; or (c) a material impairment of the prospect of repayment of any portion of the Obligations.

"**Maturity Date**" is, with respect a Term Loan, the date which is twenty-nine (29) months after the Amortization Date with respect to such Term Loan.

"**Obligations**" are Borrower's obligation to pay when due any debts, principal, interest, Lenders' Expenses, the Prepayment Fee, the Final Payment, and other amounts Borrower owes the Lenders now or later, whether under this Agreement, the Loan Documents, or otherwise, including, without limitation, all obligations relating to letters of credit (including reimbursement obligations for drawn and undrawn letters of credit), cash management services, and foreign exchange contracts, if any, and including interest accruing after Insolvency Proceedings begin (whether or not allowed) and debts, liabilities, or obligations of Borrower assigned to the Lenders and/or Collateral Agent, and the performance of Borrower's duties under the Loan Documents.

"**OFAC**" is the U.S. Department of Treasury Office of Foreign Assets Control.

"**OFAC Lists**" are, collectively, the Specially Designated Nationals and Blocked Persons List maintained by OFAC pursuant to Executive Order No. 13224, 66 Fed. Reg. 49079 (Sept. 25, 2001) and/or any other list of terrorists or other restricted Persons maintained pursuant to any of the rules and regulations of OFAC or pursuant to any other applicable Executive Orders.

"**Operating Documents**" are, for any Person, such Person's formation documents, as certified with the Secretary of State of such Person's state of formation on a date that is no earlier than 30 days prior to the Effective Date, and (a) if such Person is a corporation, its bylaws in current form, (b) if such Person is a limited liability company, its limited liability company agreement (or similar agreement), and (c) if such Person is a partnership, its

partnership agreement (or similar agreement), each of the foregoing with all current amendments or modifications thereto.

"**Oxford Equipment Financing**" is that certain Master Security Agreement dated as of July 30, 2004, by and among Borrower and Oxford and the Notes and other documents entered into from time to time prior to the Effective Date in connection therewith.

"**Payment/Advance Form**" is that certain form attached hereto as **Exhibit B**.

"**Payment Date**" is the first day of each calendar month.

"**Perfection Certificate**" is defined in Section 5.1.

"**Permitted Indebtedness**" is:

(a) Borrower's Indebtedness to the Lenders and Collateral Agent under this Agreement and the other Loan Documents;

(b) Indebtedness existing on the Effective Date and shown on the Perfection Certificate;

(c) Subordinated Debt;

(d) unsecured Indebtedness to trade creditors incurred in the ordinary course of business;

(e) Indebtedness incurred as a result of endorsing negotiable instruments received in the ordinary course of business;

(f) Indebtedness secured by Permitted Liens; and

(g) extensions, refinancings, modifications, amendments and restatements of any items of Permitted Indebtedness (a) through (f) above, provided that the principal amount thereof is not increased or the terms thereof are not modified to impose more burdensome terms upon Borrower or its Subsidiary, as the case may be.

"**Permitted Investments**" are:

(a) Investments shown on the Perfection Certificate and existing on the Effective Date; and

(b) Cash Equivalents;

(c) Investments consisting of the endorsement of negotiable instruments for deposit or collection or similar transactions in the ordinary course of Borrower;

(d) unsecured Investments to trade creditors incurred in the ordinary course of Borrower's business (e.g., up front payments under a vendor agreement); and

(e) Investments made pursuant to Borrower's Investment Policy Guidelines dated March 8, 2007, as in effect as of the Effective Date or as thereafter amended with the consent of the Required Lenders, which consent shall not be unreasonably withheld.

"**Permitted Liens**" are:

(a) Liens existing on the Effective Date and shown on the Perfection Certificate or arising under this Agreement and the other Loan Documents;

(b) Liens for taxes, fees, assessments or other government charges or levies, either not delinquent or being contested in good faith and for which Borrower maintains adequate reserves on its Books, provided that no notice of any such Lien has been filed or recorded under the Internal Revenue Code of 1986, as amended, and the Treasury Regulations adopted thereunder;

(c) purchase money Liens (i) on three pieces of laboratory Equipment acquired or held by Borrower prior to the Effective Date for financing the acquisition of the Equipment securing no more than Six Hundred Thousand Dollars ($600,000) in the aggregate amount outstanding, (ii) on Equipment acquired or held by Borrower incurred for financing the acquisition of the Equipment securing no more than One Hundred Thousand Dollars ($100,000) in the aggregate amount outstanding, or (iii) existing on Equipment when acquired, if the Lien is confined to the property and improvements and the proceeds of the Equipment;

(d) statutory Liens securing claims or demands of materialmen, mechanics, carriers, warehousemen, landlords and other Persons imposed without action of such parties, provided they have no priority over any of Collateral Agent's Lien and the aggregate amount of such Liens does not any time exceed Two Hundred Fifty Thousand Dollars ($250,000);

(e) leases or subleases of real property granted in the ordinary course of business, and leases, subleases, non-exclusive licenses or sublicenses of property (other than real property or Intellectual Property) granted in the ordinary course of Borrower's business, if the leases, subleases, licenses and sublicenses do not prohibit granting Collateral Agent a security interest;

(f) banker's liens, rights of setoff and Liens in favor of financial institutions incurred made in the ordinary course of business arising in connection with Borrower's deposit accounts or securities accounts held at such institutions to secure payment of fees and similar costs and expenses subject to Borrower's compliance with Section 6.6(b) hereof;

(g) Liens to secure payment of workers' compensation, employment insurance, old-age pensions, social security and other like obligations incurred in the ordinary course of business (other than Liens imposed by ERISA);

(h) Liens arising from judgments, decrees or attachments in circumstances not constituting an Event of Default under Section 8.5 or 8.7;

(i) non-exclusive ‘licenses of Intellectual Property granted to third parties in the ordinary course of business;

(j) exclusive licenses for the use of the property, of Borrower or its Subsidiaries in connection with joint ventures and corporate collaborations provided each such exclusive license is specifically approved by Borrower’s Board of Directors and approved with the prior written consent of the Required Lenders (which with respect to any Lender other than Oxford or MidCap shall be approved in such Lender’s reasonable discretion (other than with respect to exclusive licenses of PTC124 or PTC299 in all or substantially all the worldwide geographic regions in which the Borrower retains rights to such drug or any license which would result in a “deemed liquidation” as defined in Borrower’s Certificate of Incorporation, in respect of which such approval shall be granted in such Lender’s sole discretion));

(j) Liens in favor of Oxford or its successors or assigns encumbering Borrower’s Equipment financed pursuant to the Oxford Equipment Financing; and

(k) Liens incurred in the extension, renewal or refinancing of the indebtedness secured by Liens described in (a) and (c) above, but any extension, renewal or replacement Lien must be limited to the property encumbered by the existing Lien and the principal amount of the Indebtedness may not increase.

“**Person**” is any individual, sole proprietorship, partnership, limited liability company, joint venture, company, trust, unincorporated organization, association, corporation, institution, public benefit corporation, firm, joint stock company, estate, entity or government agency.

“**Positive Pivotal Ataluren Data**” is defined in Section 2.5 hereof.

“**Prepayment Fee**” means with respect to any Term Loan subject to prepayment prior to the Maturity Date, whether by mandatory or voluntary prepayment, acceleration or otherwise, an additional fee payable to the Lenders in amount equal to:

(i) for a prepayment made on or after the Effective Date through and including the date which is twelve (12) months thereafter, six percent (6.0%) of the principal amount of the Term Loans prepaid;

(ii) for a prepayment made after the date which is twelve (12) months after the Effective Date through and including the date which is twenty-four (24) months after the Effective Date, three and one-half percent (3.5%) of the principal amount of the Term Loans prepaid; and

(ii) for a prepayment made after the date which is twenty-four (24) months after the Effective Date and prior to the Term Loan Maturity Date, two percent (2.0%) of the principal amount of the Term Loan prepaid.

“**Pro Rata Share**” means, with respect to each Lender, a percentage (expressed as a decimal, rounded to the ninth decimal place) determined by dividing the amount of Term Loans held by such Lender by the aggregate amount of all outstanding Term Loans.

“**PTC124 Discontinuation**” is defined in Section 2.2(c)(ii).

“**Registered Organization**” is any “registered organization” as defined in the Code with such additions to such term as may hereafter be made

“**Required Lenders**” is defined in Section 12.11.

“**Requirement of Law**” is as to any Person, the organizational or governing documents of such Person, and any law (statutory or common), treaty, rule or regulation or determination of an arbitrator or a court or other Governmental Authority, in each case applicable to or binding upon such Person or any of its property or to which such Person or any of its property is subject.

“**Responsible Officer**” is any of the President and Chief Executive Officer or Chief Financial Officer of Borrower.

“**Second Draw Period**” is the period commencing on the date on which Borrower achieves Positive Pivotal Ataluren Data and ending on April 30, 2010.

“**Secured Promissory Note**” is defined in Section 2.4.

“**Secured Promissory Note Record**” is a record maintained by each Lender with respect to the outstanding Obligations and credits made thereto.

“**Securities Account**” is any “securities account” as defined in the Code with such additions to such term as may hereafter be made.

“**Subordinated Debt**” is indebtedness incurred by Borrower subordinated to all of Borrower’s now or hereafter indebtedness to the Lenders (pursuant to a subordination, intercreditor, or other similar agreement in form

and substance satisfactory to the Required Lenders entered into between Collateral Agent, Borrower and the other creditor), on terms acceptable to the Required Lenders.

“**Subsidiary**” means, with respect to any Person, any Person of which more than 50.0% of the voting stock or other equity interests (in the case of Persons other than corporations) is owned or controlled, directly or indirectly, by such Person or one or more of Affiliates of such Person.

“**Term Loan**” is defined in Section 2.2(a) hereof.

"**Term A Loan**" is defined in Section 2.2(a) hereof.

"**Term B Loan**" is defined in Section 2.2(a) hereof.

"**Term Loan Commitment**" means, for any Lender, the obligation of such Lender to make a Term Loan, up to the principal amount shown on **Schedule 1.1**. "**Term Loan Commitments**" means the aggregate amount of such commitments of all Lenders.

"**Transfer**" is defined in Section 7.1.

"**Warrants**" are those certain Warrants to Purchase Stock dated as of the Effective Date executed by Borrower in favor of each Lender.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed as of the Effective Date.

BORROWER:

PTC THERAPEUTICS, INC.

By /s/ Stuart Peltz
Name: Stuart Peltz
Title: President & CEO

COLLATERAL AGENT:

OXFORD FINANCE CORPORATION, as Collateral Agent and as a Lender

By /s/ John G. Henderson
Name: John G. Henderson
Title: Vice President & General Counsel

LENDERS:

OXFORD FINANCE CORPORATION, as a Lender

By /s/ John G. Henderson
Name: John G. Henderson
Title: Vice President & General Counsel

MIDCAP FINANCIAL, LLC, as a Lender

By /s/ Josh Groman
Name: Josh Groman
Title: Managing Director

SCHEDULE 1.1

LENDERS AND COMMITMENTS

Lender	Term A Loan Commitment		Commitment Percentage
Oxford Finance Corporation	$	10,000,000	80%
Midcap Financial, LLC	$	2,500,000	20%
TOTAL	$	12,500,000	100.00%

Lender	Term B Loan Commitment		Commitment Percentage
Oxford Finance Corporation	$	10,000,000*	80%
Midcap Financial, LLC	$	2,500,000	20%
TOTAL	$	12,500,000	100.00%

Lender	Aggregate Commitments		Commitment Percentage
Oxford Finance Corporation	$	20,000,000	80%
Midcap Financial, LLC	$	5,000,000	20%
TOTAL	$	25,000,000	100.00%

*Oxford's Term B Loan Commitment is subject to reduction in an amount equal to the aggregate outstanding obligations (including, without limitation, all principal, accrued interest and fees) owed by Borrower to Oxford in respect of the Oxford Equipment Financing on the Funding Date of Term B Loans in the event that Oxford has not sold its interest in the Oxford Equipment Financing to a third party prior to the Funding Date of Term B Loans. Oxford has no obligation to sell its interest in the Oxford Equipment Financing to a third party, and Oxford's determination of whether to sell its interest in the Oxford Equipment Financing to a third party shall be made in Oxford's sole discretion.

SCHEDULE 5.2

PTC Therapeutics, Inc.
Patent/Patent Applications
September 2009

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-104-228	Patent Cooperation Treaty	Filed	PCT/US03/19760	6/21/2003			Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC Therapeutics, Inc. ("PTC").	
1100-104-999	United States	Granted	10/519,243	12/21/2004	7,291,461	11/6/2007	Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC.	
1100-105-001	Canada	Filed	2493808.00	7/24/2003			Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC.	
1100-105-227	European Patent Convention	Filed	3765998.00	7/24/2003			Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC.	
1100-105-228	Patent Cooperation Treaty	Filed	PCT/US03/23075	7/24/2003			Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC.	
1100-105-999	United States	Filed	10/521,775	1/21/2005			Methods for Identifying Small Molecules that Modulate Premature Translation Termination and Nonsense	Owned by PTC.	
1100-109-001	Canada	Filed	2493816.00	7/23/2003			Use of Nucleoside Compounds and their Use for Nonsense Suppression and the Treatment of Genetic Dise	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-109-227	European Patent Convention	Filed	3766015.60	7/23/2003			Use of Nucleoside Compounds and their Use for Nonsense Suppression and the Treatment of Genetic Dise	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-109-228	Patent Cooperation Treaty	Filed	PCT/US03/23185	7/23/2003			Use of Nucleoside Compounds and their Use for Nonsense Suppression and the Treatment of Genetic Dise	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-109-999	United States	Granted	11/048,659	1/21/2005	7,449,570	11/11/2008	Use of Nucleoside Compounds for Nonsense Suppression and the Treatment of Genetic Diseases	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-110-001	Canada	Filed	2493458.00	1/25/2005			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-110-007	Australia	Filed	256755.00	7/23/2003			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-110-227	European Patent Convention	Filed	3766012.00	7/23/2003			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-110-228	Patent Cooperation Treaty	Filed	PCT/US03/23182	7/23/2003			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-110-777	United States	Filed	TBD	4/17/2008			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-110-999	United States	Granted	11/048.656	1/21/2005	7,405,233	7/29/2008	Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1100-111-001	Canada	Filed	2493457.00	7/23/2003			Acetylamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of Disea	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-111-007	Australia	Filed	254157.00	7/23/2003			Acetylamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of Disea	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-111-227	European Patent Convention	Filed	3766013.00	7/23/2003			Acetylamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of Disea	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-111-228	Patent Cooperation Treaty	Filed	PCT/US03/23183	7/23/2003			Acetylamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of Disea	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-111-777	United States	Filed	11/789,625	4/24/2007			Use of Acetylamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-111-999	United States	Granted	11/048,657	1/21/2005	7,247,741	7/24/2007	Acetytamino Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of Disea	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.	
1100-118-001	Canada	Filed	2521992.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-007	Australia	Filed	2004229487.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-008	New Zealand	Granted	543263.00	4/9/2004	543263	4/9/2009	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-009	Mexico	Filed	2005/010747	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	

1100-118-012	Japan	Filed	2006-509896	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-015	Norway	Filed	20055314.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-019	Russian Federation	Granted	200501601.00	4/9/2004	9120	8/22/2007	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-020	Cuba	Filed	190/2005	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-023	Hong Kong	Filed	7100245.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-026	Colombia	Filed	5113348.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-037	Brazil	Filed	PI0409319-4	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-055	Singapore	Granted	200506657-6	4/9/2004	118461	10/31/2007	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.
1100-118-056	India	Granted	2948/CHENP/2005	4/9/2004	230604	5/25/2009	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-118-070	Costa Rica	Filed	8086.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-076	Indonesia	Filed	W-00200503004	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-078	Egypt	Filed	633/2005	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-106	Morocco	Granted	28594.00	4/9/2004	27802	4/9/2004	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-117	Philippines	Filed	1-2005-501823	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-139	Trinidad	Filed	TT/VA/2005/00165	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-146	China P.R.	Filed	200480015905.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-147	South Africa	Granted	2005/08298	4/9/2004	2005/08298	1/31/2007	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-158	Israel	Filed	171343.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-187	Korea South	Filed	10-2005-7019319	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-200	Eurasian Patent Convention	Granted	200501601.00	4/9/2004	9120	8/22/2007	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-220	United Arab Emirates	Filed	270/2006	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-227	European Patent Convention	Filed	4759404.90	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-228	Patent Cooperation Treaty	Filed	PCT/US04/11106	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-255	Ukraine	Filed	200510644.00	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-259	Mongolia	Granted	3641.00	4/9/2004	2707	4/21/2006	1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	
1100-118-280	Uzbekistan	Filed	IAP20050393	4/9/2004			1,2,4 Oxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-118-555	United States	Granted	11/241,700	9/29/2005	7,202,262	4/10/2007	Benzoic Acid or Benzonate Substituted 1,2,4-Oxadiazole Compounds and Their Use For the Treatment of	Owned by PTC.	Ataluren - CoM
1100-118-777	United States	Filed	11/042,652	1/24/2005			1,2,4 Qxadiazole Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	Ataluren - MoU
1100-118-999	United States	Granted	10/822,259	4/9/2004	6,992,096	1/31/2006	1,2,4 Oxadiazote Benzoic Acid Compounds and their Use for	Owned by PTC.	Ataluren - CoM

							Nonsense Suppression and the Treatment of	
1100-134-001	Canada	Filed	2394470.00	7/14/2002			Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-007	Australia	Granted	22720/01	12/13/2001	784947	6/3/2006	Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-012	Japan	Filed	2001-545593	6/14/2002			Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-014	France	Granted	986492.70	12/13/2000	1238110	2/18/2009	Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-017	Italy	Granted	986492.70	12/13/2000	1238110	2/18/2009	Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-018	Switzerland	Granted	986492.70	12/13/2000	1238110	2/18/2009	Methods of Assaying for Compounds that Inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-698	Germany	Granted	986492.70	12/13/2000	1238110	2/18/2009	Methods of Assaying for Compounds that Inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-227	European Patent Convention	Granted	986492.70	12/13/2000	1238110	2/18/2009	Methods of Assaying for Compounds that Inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-228	Patent Cooperation Treaty	Filed	PCT/US00/34232	12/13/2000			Methods of Assaying for Compounds that Inhibit Premature Translation Termination and Nonsense-mediat	Jointly owned by PTC and Amgen SF, LLC. Exclusively licensed to PTC pursuant to the Tularik Agreement.
1100-134-666	United States	Granted	10/228,803	8/21/2002	7,026,122	4/11/2006	Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-Mediat	Ownership assigned to PTC pursuant to the Tularik Agreement.	Formerly jointly owned by PTC and Tularik
1100-134-777	United States	Filed	11/318,940	12/22/2005			Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Ownership assigned to PTC pursuant to the Tularik Agreement.	Formerly jointly owned by PTC and Tularik
1100-134-999	United States	Granted	09/461,508	12/14/1999	6,458,538	10/1/2002	Methods of Assaying for Compounds that Inhibit Premature Translation Termination and Nonsense-mediat	Ownership assigned to PTC pursuant to the Tularik Agreement.	Formerly jointly owned by PTC and Tularik
1100-207-227	European Patent Convention	Filed	8019547.20	11/6/2008			Methods of Assaying for Compounds that inhibit Premature Translation Termination and Nonsense-mediat	Owned by PTC.	
1100-138-001	Canada	Filed	2493815.00	7/23/2003			Nucleoside Compounds and their use for Treating Cancer and Diseases Associated with Somatic Mutation	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-138-227	European Patent Convention	Filed	3766014.90	7/23/2003			Nucleoside Compounds and their use for Treating Cancer and Diseases Associated with Somatic Mutation	Owned by PTC.	
1100-138-228	Patent Cooperation Treaty	Filed	PCT/US03/23184	7/22/2003			Nucleoside Compounds and their use for Treating Cancer and Diseases Associated with Somatic Mutation	Owned by PTC.	
1100-138-777	United States	Filed	11/906,665	10/2/2007			Nucleoside Compounds and their use for Treating Cancer and Diseases Associated with Somatic Mutation	Owned by PTC.	
1100-138-999	United States	Granted	10/625,059	7/22/2003	7,291,603	11/6/2007	Nucleoside Compounds and their use for Treating Cancer and Diseases Associated with Somatic Mutation	Owned by PTC.	
1100-151-001	Canada	Filed	2582885.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-151-227	European Patent Convention	Filed	5804171.60	10/13/2005			Compounds for Nonsense Suppression Use of These Compounds for the Manufacture of a Medicament for Tr	Owned by PTC.	
1100-151-228	Patent Cooperation Treaty	Filed	PCT/US05/036762	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-151-999	United States	Filed	11/577,176	10/13/2005			Compounds for Nonsense Suppression, Use of These Compounds for the Manufacture of a Medicament For	Owned by PTC.	
1100-152-001	Canada	Filed	2583976.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-152-007	Australia	Filed	2005296730.00	10/13/2005			Use of Defined Compounds for the Manufacture of a Medicament for Preventing/Treating Diseases Result	Owned by PTC.	
1100-152-008	New Zealand	Filed	554310.00	10/13/2005			Use of Defined Compounds for the Manufacture of a Medicament for Preventing/Treating Diseases Result	Owned by PTC.	
1100-152-009	Mexico	Filed	MX/a/2007/004487	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-152-012	Japan	Filed	2007-536867	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-152-023	Hong Kong	Filed	7114098.50	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-152-056	India	Filed	3575/DELNP/2007	10/13/2005			Use of Defined Compounds for the Manufacture of a	Owned by PTC.	

							Medicament for Preventing/Treating Diseases Result		
1100-152-146	China P.R.	Filed	200580042808.50	10/13/2005			Use of Defined Compounds for the manufacture of a Medicament for Preventing/Treating Diseases Result	Owned by PTC.	
1100-152-158	Israel	Filed	182461.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-152-227	European Patent Convention	Filed	5807462.60	10/13/2005			Use of Defined Compounds for the Manufacture of a Medicament for Preventing/Treating Diseases Result	Owned by PTC.	
1100-152-228	Patent Cooperation Treaty	Filed	PCT/US05/36764	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-152-461	Korea South	Filed	10-2007-7009154	10/13/2005			Use of Defined Compounds for the Manufacture of a Medicament for Preventing/Treating Diseases Result	Owned by PTC.	
1100-152-999	United States	Filed	11/577,189	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-153-001	Canada	Filed	2583177.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-153-227	European Patent Convention	Filed	5807667.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-153-228	Patent Cooperation Treaty	Filed	PCT/US05/037052	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-153-999	United States	Filed	11/577,192	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-154-001	Canada	Filed	2583971.00	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-007	Australia	Filed	2005295727.00	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-008	New Zealand	Filed	554437.00	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-009	Mexico	Filed	MX/a/2007/004484	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-012	Japan	Filed	2007-536865	10/13/2005			Pyrazole or Triazole Compounds and Their Use for The Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-023	Hong Kong	Filed	7114097.60	12/27/2007			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-037	Brazil	Filed	P10515995-4	10/13/2005			Pyrazole or Triazote Compounds and Their USe for the Manufacture of Medicament for Treating Somatic	Owned by PTC.	
1100-154-055	Singapore	Filed	200702648-7	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-056	India	Filed	3576/DELNP/2007	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-117	Phillpines	Filed	1-2007-500807	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-154-146	China P.R.	Filed	200580042744.90	10/13/2005			Pyrazoles or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-147	South Africa	Filed	2007/03671	10/13/2005			COMPOUNDS FOR NONSENSE SUPPRESSION, AND METHODS FOR THEIR USE\n	Owned by PTC.	
1100-154-158	Israel	Filed	182464.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-154-227	European Patent Convention	Filed	5804194.80	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-228	Patent Cooperation Treaty	Filed	PCT/US05/036761	10/13/2005			Compounds for Nonsense Suppression, and Methods for their Use	Owned by PTC.	
1100-154-461	Korea South	Filed	10-2007-7010794	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a Medicament for Treating Somatic	Owned by PTC.	
1100-154-999	United States	Filed	11/577,177	10/13/2005			Pyrazole or Triazole Compounds and Their Use for the Manufacture of a	Owned by PTC.	

							Medicament for Treating Somatic		
1100-157-001	Canada	Filed	2583159.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-007	Australia	Filed	2005295778.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-008	New Zealand	Filed	554327.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-009	Mexico	Filed	MX/a/2007/004479	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-012	Japan	Filed	2007-536837	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-023	Hong Kong	Filed	8101510.10	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-037	Brazil	Filed	PI0516110-0	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-055	Singapore	Filed	200702507-5	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-056	India	Filed	3577/DELNP/2007	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-117	Philippines	Filed	1-2007-500805	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-146	China P.R.	Filed	200580042743.40	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-147	South Africa	Filed	2007/02933	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-158	Israel	Filed	182459.00	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-227	European Patent Convention	Filed	5815159.80	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-228	Patent Cooperation Treaty	Filed	PCT/US05/036673	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-461	Korea South	Filed	10-2007-7010767	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-157-999	United States	Filed	11/577,191	10/13/2005			Compounds for Nonsense Suppression, and Methods for Their Use	Owned by PTC.	
1100-170-001	Canada	Filed	2603402.00	4/6/2006			Compositions of an Orally Active 1,2,4-Oxadiazole for Nonsense Mutuation Suppression Therapy	Owned by PTC.	
1100-170-007	Australia	Filed	2006235115.00	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Suppress	Owned by PTC.	
1100-170-008	New Zealand	Filed	562197.00	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-170-009	Mexico	Filed	MX/a/2007/012206	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-012	Japan	Filed	2008-505534	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-015	Norway	Filed	20075682.00	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-023	Hong Kong	Filed	8106626.10	6/17/2008			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-037	Brazil	Filed	PI0609089-3	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-055	Singapore	Filed	200716713-3	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadlaiofe for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-076	Indonesia	Filed	W-00200703359	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-146	China P.R.	Filed	200680020100.40	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-158	Israel	Filed	186433.00	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadllazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-227	European Patent Convention	Filed	6749439.30	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadllazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-228	Patent Cooperation Treaty	Filed	PCT/US06/012887	4/6/2006			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-170-999	United States	Filed	11/918,114	10/4/2007			Compositions and Methods for Dosing an Orally Active 1,2,4-oxadiazoie for Nonsense Mutation Therapy	Owned by PTC.	Ataluren - MoU Low Dosing Regimen
1100-171-777	United States	Granted	11/370,229	3/6/2006	7,304,080	12/74/2007	Substituted 1,2,4-Oxadiazoles, Compositions and Methods of Use	Owned by PTC.	
1100-173-007	Australia	Filed	2007235524.00	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-008	New Zealand	Filed	571899.00	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-009	Mexico	Filed	Mx/a/2008/012515	3/29/2007			Methods for Production of Functional Protein from DNA	Owned by PTC.	

							Having a Nonsense Mutation and the Treatment		
1100-173-012	Japan	Filed	TBD	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-015	Norway	Filed	20084488.00	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-173-019	Russian Federation	Filed	2007142957.00	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-037	Brazil	Filed	PI0710213-5	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-055	Singapore	Filed	200807301-7	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-056	India	Filed	5887/CHENP/2008	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-076	Indonesia	Filed	W00200803279	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-146	China P.R.	Filed	200780020127.80	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-158	Israel	Filed	194454.00	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-227	European Patent Convention	Filed	7754745.30	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-228	Patent Cooperation Treaty	Filed	PCT/US07/008268	3/29/2007			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	
1100-173-999	United States	Filed	12/234,592	9/29/2008			Methods for Production of Functional Protein from DNA Having a Nonsense Mutation and the Treatment	Owned by PTC.	Ataluren - MoU - Novel Protein
1100-174-666	United States	Granted	11/370,130	3/6/2006	7,419,991B2	9/2/2008	3-[5-(2-Fluoro-Phenyt-[1,2,4]Oxadlazol-3-YL]-Benzoic Acid, Compositions, and Methods for the Use The	Owned by PTC.	Ataluren - Picture Claim - CoM
1100-175-001	Canada	Filed	2662749.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-002	Argentina	Filed	P070103975	9/7/2007			Processes for the Preparation of 1,2,4-Oxadiazoie Benzoic Acids	Owned by PTC.	
1100-175-003	Chile	Filed	2606-2007	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-004	Malaysia	Filed	PI20090941	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazoie Benzoic Acids	Owned by PTC.	
1100-175-005	Peru	Filed	1203-2007	9/7/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-007	Australia	Filed	2007292915.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-008	New Zealand	Filed	575511.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-009	Mexico	Filed	MX/a20O9/002439	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazoie Benzoic Acids	Owned by PTC.	
1100-175-011	Taiwan	Filed	96133607.00	9/8/2006			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-012	Japan	Filed	2009-527430	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-015	Norway	Filed	TBD	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-019	Russian Federation	Filed	2009113019.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazoie Benzoic Acids	Owned by PTC.	
1100-175-023	Hong Kong	Filed	TBD	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	

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1100-175-037	Brazil	Filed	PI0716996-5	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-055	Singapore	Filed	200901588-4	9/6/2007			Processes for the Preparation of 1,2,4-Oxadlazole Benzoic Acids	Owned by PTC.	
1100-175-056	India	Filed	TBD	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-117	Philippines	Filed	1-2009-500433	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazote Benzoic Acids	Owned by PTC.	
1100-175-255	Ukraine	Filed	200903350.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadlazole Benzoic Acids	Owned by PTC.	
1100-175-146	China P.R.	Filed	200780041169.X	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-147	South Africa	Filed	2009/01782	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-158	Israel	Filed	197445.00	9/6/2007			Processes for the Preparation of 1,2,4-Oxadlazole Benzoic Acids	Owned by PTC.	
1100-175-187	Korea South	Filed	10-2009-7007202	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-189	Vietnam	Filed	1-2009-00626	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-227	European Patent Convention	Filed	7837899.90	9/6/2007			Processes for the Preparation of 1,2,4-Oxadlazole Benzoic Acids	Owned by PTC.	
1100-175-228	Patent Cooperation Treaty	Filed	PCT/US07/019561	9/6/2007			Processes for the Preparation of 1,2,4-Oxadiazole Benzoic Acids	Owned by PTC.	
1100-175-999	United States	Filed	11/899,813	9/6/2007			Processes for the Preparation of 1,2,4-Qxadlazote Benzoic Acids	Owned by PTC.	Ataluren - Manufacturing Process
1100-177-001	Canada	Filed	2663574.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-002	Argentina	Filed	P070104212	9/24/2007			Crystalline Forms of a 3-[5-(2-Dluorophenyl)-[1,2,4]Oxadlazole-3-YL]-Benzoic Acid	Owned by PTC.	

1100-177-003	Chile	Filed	2743-2007	9/25/2006			Crystalline Forms of a 3-[5-(2-Dluorophenyl)-[1,2,4]Oxadlazole-3-YL]-Benzoic Acid	Owned by PTC.	
1100-177-004	Malaysia	Filed	PI20091186	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazoie-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-005	Peru	Filed	1290.20	9/25/2007			Crystalline Forms of a 3-[5-(2-Dluorophenyl)-[1,2,4]Oxadiazole-3-YL]-Benzoic Acid	Owned by PTC.	
1100-177-007	Australia	Filed	2007300542.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-008	New Zealand	Filed	575795.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-009	Mexico	Filed	MX/a/2009/003160	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-011	Taiwan	Filed	96135785.00	9/26/2007			Crystalline Forms of a 3-[5-(2-Dluorophenyl)-[1,2,4]Oxadiazole-3-YL]-Benzoic Acid	Owned by PTC.	
1100-177-012	Japan	Filed	2009-529270	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-013	Venezuela	Filed	2007-002061	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1100-177-015	Norway	Filed	20091488.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-019	Russian Federation	Filed	2009115649.00	9/24/2007			Crystalline Forms of 3-(5-(2-Fluorophenyl)-[1,2,4]-Oxadiazote-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-037	Brazil	Filed	PI0717107-2	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadlazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-055	Singapore	Filed	200901948-0	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-076	Indonesia	Filed	W-00200900747	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadlazote-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-117	Philippines	Filed	1-2009-500530	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadlazole-3-Yt]- Benzoic Acid	Owned by PTC.	
1100-177-146	China P.R.	Filed	200780043582.X	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-147	South Africa	Filed	2009/01949	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-158	Israel	Filed	197717.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4)-Oxadiazote-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-187	Korea South	Filed	10-2009-7008202	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-189	Vietnam	Filed	1-2009-00809	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-227	European Patent Convention	Filed	7838770.10	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-228	Patent Cooperation Treaty	Filed	PCT/US07/020633	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazote-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-255	Ukraine	Filed	200904012.00	9/24/2007			Crystalline Forms of 3-[5-(2-Fluorophenyl)-[1,2,4]-Oxadiazole-3-YL]- Benzoic Acid	Owned by PTC.	
1100-177-999	United States	Filed	11/904,005	9/24/2007			Crystalline Forms of a 3-[5-(2-Dluorophenyl)-[1,2,4]Oxadiazole-3-YL]-Benzoic Add	Owned by PTC.	Ataluren - Solid Forms
1100-178-227	European Patent Convention	Filed	7873433.20	9/24/2007			Hydroxylated 1,2,4-Oxadiazole Benzoic Acid Compounds, Compositions Thereof and the Use for Nonsense	Owned by PTC.	
1100-178-228	Patent Cooperation Treaty	Filed	PTC/US07/020631	9/24/2007			Hydroxylated 1,2,4-Oxadiazole Benzoic Acid Compounds, Compositions Thereof and the Use for Nonsense	Owned by PTC.	
1100-178-999	United States	Filed	11/904,001	9/24/2007			Hydroxylated 1,2,4-Oxadiazole Benzoic Acid Compounds, Compositions Thereof and the Use for Nonsense	Owned by PTC.	Ataluren - Metabolites
1100-180-777	United States	Filed	11/724,408	3/14/2007			1,2,4-Oxadiazote Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment of	Owned by PTC.	Ataluren - Pharmaceutical Compositions
1100-181-003	Chile	Filed	2923-2007	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	

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1100-181-005	Peru	Filed	1386-2007	10/12/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-007	Australia	Filed	2007308067.00	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-009	Mexico	Filed	MX/a/2009/003909	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-011	Taiwan	Filed	96138340.00	10/12/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-012	Japan	Filed	2009-532453	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-015	Norway	Filed	20091826.00	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-019	Russian Federation	Filed	2009117590.00	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-055	Singapore	Filed	200902485-2	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-076	Indonesia	Filed	W-00200900954	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-146	China P.R.	Filed	200780045655.00	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-187	Korea South	Filed	10-2009-7009602	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-227	European Patent Convention	Filed	7852738.90	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-228	Patent Cooperation Treaty	Filed	PCT/US07/021921	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	
1100-181-999	United States	Filed	11/974,068	10/11/2007			Methods for Dosing an Orally Active 1,2,4-Oxadiazole for Nonsense Mutation Therapy	Owned by PTC.	Ataluren - High Dosing
1100-204-777	United States	Filed	12/140,641	6/17/2008			Ureido Substituted Benzoic Acid Compounds and their Use for Nonsense Suppression and the Treatment o	Owned by PTC.	
1200-102-001	Canada	Filed	2443711.00	10/8/2003			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-102-012	Japan	Filed	581690.00	4/11/2002			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-102-227	European Patent Convention	Filed	2725663.50	4/11/2002			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-102-228	Patent Cooperation Treaty	Filed	PCT/US02/11757	4/11/2002			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-102-666	United States	Filed	11/347,748	2/3/2006			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-102-999	United States	Filed	10/475,024	2/3/2004			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	

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1200-103-228	Patent Cooperation Treaty	Filed	PCT/US02/11758	4/11/2002			Methods for identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-103-777	United States	Filed	11/059,721	2/21/2006			Methods for Identifying Small Molecules that Bind Specific RNA Structural Motifs	Owned by PTC.	
1200-113-001	Canada	Filed	2520415.00	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-113-012	Japan	Filed	2006-509431	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-113-227	European Patent Convention	Filed	4758530.20	9/26/2005			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-113-228	Patent Cooperation Treaty	Filed	PCT/US04/009572	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-113-999	United States	Filed	10/551,301	9/27/2005			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-114-001	Canada	Filed	2520510.00	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-114-012	Japan	Filed	2006-509432	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-114-227	European Patent Convention	Filed	4758532.00	9/25/2005			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-114-228	Patent Cooperation Treaty	Filed	PCT/US04/009574	3/26/2004			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-114-999	United States	Filed	10/551,304	9/27/2005			Methods for Identifying Compounds that Target tRNA Splicing Endonuclease and Uses of Said Compounds	Owned by PTC.	
1200-115-001	Canada	Filed	2520664.00	3/26/2004			Targeting Enzymes of the tRNA Splicing Pathway for Identification of Anti-Fungal And/Or Anti-Prolife	Owned by PTC.	
1200-115-012	Japan	Filed	2006-509407	3/26/2004			Targeting Enzymes of the tRNA Splicing Pathway for Identification of Anti-Fungal And/Or Anti-Prolife	Owned by PTC.	
1200-115-227	European Patent Convention	Filed	4758540.10	9/25/2005			Targeting Enzymes of the tRNA Splicing Pathway for Identification of Anti-Fungal And/Or Anti-Prolife	Owned by PTC.	
1200-115-228	Patent Cooperation Treaty	Filed	PCT/US04/009590	3/26/2004			Targeting Enzymes of the tRNA Splicing Pathway for identification of Anti-Fungal And/Or Antl-Proiife	Owned by PTC.	
1200-115-999	United States	Filed	10/551.300	9/27/2005			Targeting Enzymes of the tRNA	Owned by PTC.	

							Splicing Pathway for Identification of Anti-Fungal And/Or Anti-Prolife		
1200-121-001	Canada	Filed	2574076.00	8/16/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMS II)	Owned by PTC.	
1200-121-012	Japan	Filed	2007-522473	8/16/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMS II)	Owned by PTC.	

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1200-121-023	Hong Kong	Filed	7107669.80	8/16/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMS II)	Owned by PTC.	
1200-121-227	European Patent Convention	Filed	4781055.10	8/16/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMS II)	Owned by PTC.	
1200-121-228	Patent Cooperation Treaty	Filed	PCT/US04/26309	8/16/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMS II)	Owned by PTC.	
1200-121-999	United States	Filed	10/895.393	7/21/2004			Methods and Agents for Screening for Compounds Capable of Modulating Gene Expression (GEMSII)	Owned by PTC.	GEMS
1200-122-001	Canada	Filed	2514184.00	1/21/2004			Methods for Identifying Compounds that Modulate Untranslated Region-Based Regulation and Methods of	Owned by PTC.	
1200-122-227	European Patent Convention	Filed	4704085.20	1/21/2004			Methods for Identifying Compounds that Modulate Untranslated Region-Based Regulation and Methods of	Owned by PTC.	
1200-122-228	Patent Cooperation Treaty	Filed	PCT/US04/001643	1/21/2004			Methods for Identifying Compounds that Modulate Untranslated Region-Based Regulation and Methods of	Owned by PTC.	
1200-122-999	United States	Filed	10/543,033	7/21/2005			Methods for identifying Compounds that Modulate Untranslated Region-Based Regulation and Methods of	Owned by PTC.	GEMS
1200-140-001	Canada	Filed	2531321.00	7/2/2004			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-140-012	Japan	Filed	2006-518793	7/2/2004			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-140-227	European Patent Convention	Filed	4777457.70	7/2/2004			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-140-228	Patent Cooperation Treaty	Filed	PCT/US04/021334	7/2/2004			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-140-666	United States	Filed	12/317,899	12/30/2008			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-140-999	United States	Filed	10/884,695	7/2/2004			RNA Processing Protein Complexes and Uses Thereof	Owned by PTC.	
1200-141-227	European Patent Convention	Filed	TBD	1/17/2004			Methods and Systems for the Identification of RNA Regulatory Sequences and Compounds that Modulate t	Owned by PTC.	
1200-141-228	Patent Cooperation Treaty	Filed	PCT/US04/000423	1/17/2004			Methods and Systems for the Identification of RNA Regulatory Sequences and Compounds that Modulate t	Owned by PTC.	
1200-141-999	United States	Filed	10/542,255	7/17/2005			Methods and Systems for the Identification of RNA Regulatory Sequences and Compounds that Modulate t	Owned by PTC.	
1200-145-001	Canada	Filed	2546363.00	5/17/2006			Methods and Agents for Screening for Compounds Capable of Modulating Her2 Expression	Owned by PTC.	
1200-145-227	European Patent Convention	Filed	4811268.40	11/17/2004			Methods and Agents for Screening for Compounds Capable of Modulating Her2 Expression	Owned by PTC.	
1200-145-228	Patent Cooperation Treaty	Filed	PCT/US04/38496	11/17/2004			Methods and Agents for Screening for Compounds Capable of Modulating Her2 Expression	Owned by PTC.	
1200-145-999	United States	Filed	10/579,500	5/16/2006			Methods and Agents for Screening for Compounds Capable of Modulating Her2 Expression	Owned by PTC.	

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1200-160-228	Patent Cooperation Treaty	Filed	PCT/US05/047156	12/28/2005			Cell Based Methods and Systems for the Identification of RNA Regulatory Sequences and Compounds that	Owned by PTC.	
1200-160-999	United States	Filed	11/813,027	12/28/2005			Cell Based Methods and Systems for the Identification of RNA Regulatory Sequences and Compounds that	Owned by PTC.	
1200-189-999	United States	Filed	12/143,705	6/20/2008			Methods for Treating Muscular Dystrophy (IGF1 - Alpha 7 - Utrophin - GEMS)	Owned by PTC.	
1200-190-999	United States	Filed	12/143,697	6/20/2008			Methods for Treating Muscular Dystrophy (Myostatin - GEMS)	Owned by PTC.	
1200-191-999	United States	Filed	12/144,577	6/23/2007			Methods for Treating Spinal Muscular Atrophy (SMA GEMS)	Owned by PTC.	
1200-202-228	Patent Cooperation Treaty	Filed	PCT/US09/003238	5/27/2009			Methods for Treating Spinal Muscular Atrophy	Owned by PTC.	
1200-202-999	United States	Filed	12/473,116	5/27/2009			Methods for Treating Spinal Muscular Atrophy	Owned by PTC.	
1200-205-228	Patent Cooperation Treaty	Filed	OCT/US09/004636	8/13/2009			Methods For Treating Viral Infections	Owned by PTC.	
1200-206-228	Patent Cooperation Treaty	Filed	PCT/US09/004625	8/13/2009			Methods For Treating Muscular Athropies	Owned by PTC.	
1200-206-888	United States	Filed	61/156,429	2/27/2009			Methods For Treating Muscular Athropies	Owned by PTC.	
1300-146-001	Canada	Filed	2588389.00	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-009	Mexico	Filed	MX/a/2007/006178	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	

1300-146-012	Japan	Filed	2007-543448	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-023	Hong Kong	Filed	8101584.20	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-146	China P.R.	Filed	200580046708X	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-227	European Patent Convention	Filed	5852074.30	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-228	Patent Cooperation Treaty	Filed	PCT/US2005/042482	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-146-999	United States	Filed	11/720,061	11/23/2005			Substituted Phenols as Active Agents Inhibiting VEGF Production	Owned by PTC.	
1300-147-001	Canada	Filed	2559408.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Anglogenesis	Owned by PTC.	
1300-147-007	Australia	Filed	2005222632.00	3/15/2005			Carboline Derivatives Useful in the inhibition of Angiogenesis	Owned by PTC.	
1300-147-008	New Zealand	Filed	550258.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Anglogenesis	Owned by PTC.	
1300-147-009	Mexico	Filed	PA/a/2006/010546	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-012	Japan	Filed	2007-504011	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-015	Norway	Filed	2006 4519	3/15/2005			Carboline Derivatives Useful In the inhibition of Angiogenesis	Owned by PTC.	
1300-147-023	Hong Kong	Filed	7107009.70	6/29/2007			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-026	Colombia	Filed	6104127.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-037	Brazil	Filed	TBD	3/15/2005			Carbcline Derivatives Useful in the Inhibition of Anglogenesis	Owned by PTC.	
1300-147-055	Singapore	Filed	200606424-0	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-056	India	Filed	6031/DELNP/2006	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	

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1300-147-068	Ecuador	Filed	SP-06-6928	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-076	Indonesia	Filed	WO200602552	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-117	Philippines	Filed	12006501796.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-146	China P.R.	Filed	200580014943.90	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-147	South Africa	Granted	2006/08176	3/15/2005	2006/08176	8/28/2008	Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-158	Israel	Filed	178079.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-200	Eurasian Patent Convention	Filed	100601697.00	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-227	European Patent Convention	Filed	5725560.60	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-228	Patent Cooperation Treaty	Filed	PCT/US05/008481	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-255	Ukraine	Filed	2006 10306	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-461	Korea South	Filed	10-2006-7021300	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-147-999	United States	Filed	11/079,420	3/15/2005			Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	PTC299 - CoM
1300-148-001	Canada	Filed	2559545.00	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-007	Australia	Filed	2005222627.00	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful In the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-009	Mexico	Filed	PA/A/2006/010542	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-012	Japan	Filed	2007-503999	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-023	Hong Kong	Filed	7106283.60	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful In the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-146	China P.R.	Filed	200580014951.30	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-227	European Patent Convention	Filed	5733369.20	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-228	Patent Cooperation Treaty	Filed	PCT/US05/08452	3/15/2005			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-148-999	United States	Filed	10/592,761	9/14/2006			Tetra-Cyclic Carboline Derivatives Useful in the Inhibition of Angiogenesis	Owned by PTC.	
1300-149-001	Canada	Filed	2567111.00	6/28/2004			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	
1300-149-012	Japan	Filed	TBD	6/28/2004			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	
1300-149-023	Hong Kong	Filed	7106179.30	6/11/2007			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	
1300-149-227	European Patent Convention	Filed	4809465.00	6/28/2004			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	
1300-149-228	Patent Cooperation Treaty	Filed	PCT/US04/020751	6/28/2004			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	

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1300-149-999	United States	Filed	10/851,074	5/24/2004			Methods and Agents for Screening for Compounds Capable of Modulating VEGF Expression	Owned by PTC.	GEMS - Biotech

1300-156-001	Canada	Filed	2588607.00	11/23/2005			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-009	Mexico	Filed	MX/a/2007/006180	11/23/2005			Carbazole, Carboline. and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-012	Japan	Filed	2007-543450	11/23/2005			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-023	Hong Kong	Filed	TBD	7/16/2007			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-146	China P.R.	Filed	200580046669.30	7/16/2007			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-227	European Patent Convention	Filed	5852076.80	11/23/2005			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-228	Patent Cooperation Treaty	Filed	PCT/US2005/042484	11/23/2005			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-156-999	United States	Filed	11/720,057	11/23/2005			Carbazole, Carboline, and the Indole Derivatives Useful in the Inhibition of VEGF Production	Owned by PTC.	
1300-158-001	Canada	Filed	2588384.00	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-009	Mexico	Filed	MX/a/2007/006179	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-012	Japan	Filed	2007-543449	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-023	Hong Kong	Filed	8101585.10	11/23/2005			Tetrahydrocarbazoles as Active Agents for inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-146	China P.R.	Filed	200580046673.X	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-227	European Patent Convention	Filed	5852075.00	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-228	Patent Cooperation Treaty	Filed	PCT/US2005/042483	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-158-999	United States	Filed	11/720.055	11/23/2005			Tetrahydrocarbazoles as Active Agents for Inhibiting VEGF Production by Translational Control	Owned by PTC.	
1300-166-012	Japan	Filed	2008-506824	4/17/2006			Carboline Derivatives Useful in the Treatment of Cancer	Owned by PTC.	
1300-166-023	Hong Kong	Filed	8107284.20	4/17/2006			Carboline Derivatives Useful in the Treatment of Cancer	Owned by PTC.	
1300-166-227	European Patent Convention	Filed	6750554.50	4/17/2006			Carboline Derivatives Useful in the Treatment of Cancer	Owned by PTC.	
1300-166-228	Patent Cooperation Treaty	Filed	PCT/US06/014547	4/17/2006			Carboline Derivatives Useful in the Treatment of Cancer	Owned by PTC.	
1300-166-333	United States	Filed	11/107,783	4/18/2005			Carboline Derivatives Useful in the Treatment of Cancer	Owned by PTC.	PTC299 - MoU
1300-172-999	United States	Filed	11/765,871	6/20/2007			Inhibition of VEGF Translation	Owned by PTC.	PTC299 - MoU

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1300-185-228	Patent Cooperation Treaty	Filed	PCT/US08/004810	4/12/2008			Administration of Carboline Derivatives Useful in the Treatment of Cancer and other Diseases	Owned by PTC.	
1300-185-333	United States	Filed	11/735.069	4/13/2007			Administration of Carboline Derivatives Useful in the Treatment of Cancer and other Diseases	Owned by PTC.	PTC299 - MoU
1300-186-228	Patent Cooperation Treaty	Filed	PCT/US08/004809	4/12/2008			Administration of Carboline Derivatives Useful In the Treatment of Cancer and Other Diseases	Owned by PTC.	
1300-194-228	Patent Cooperation Treaty	Filed	PCT/US09/43395	7/1/2009			BMI-1 Protein Expression Modulators	Owned by PTC.	
1300-195-228	Patent Cooperation Treaty	Filed	PCT/US2009/049399	7/1/2009			Methods for Screening for Compounds for Treating Cancer BMI-1)	Owned by PTC.	
1300-197-888	United States	Docketed					Carbolines Derivatives Associating with the VDAC and other proteins and the Use of Carboline Derivat	Owned by PTC.	
1300-198-888	United States	Docketed					SIRNA Molecules for the Treatment of Cancer and other Diseases	Owned by PTC.	
1300-199-888	United States	Docketed					Carbolines Derivatives Useful in the Treatment of Cancer and other Diseases (CIP 868)	Owned by PTC.	
1300-213-888	United States	Filed	61/181,651	5/27/2009			Method of Treating Kaposi Sarcoma	Owned by PTC.	
1300-214-888	United States	Filed	61/181,649	5/27/2009			Methods for Treating Prostate Conditions \n	Owned by PTC.	
1300-215-888	United States	Filed	61/181,652	5/27/2009			Processes for the Preparation of Substituted Tetrahydro Beta-Carbolines	Owned by PTC.	
1300-216-888	United States	Filed	61/181,653	5/27/2009			Methods for Treating Cancer and Non-Neoplastic Conditions	Owned by PTC.	
1300-217-888	United States	Filed	61/181,654	5/27/2009			Methods for Treating Brain Tumors	Owned by PTC.	
1300-218-888	United States	Filed	61/181,650	5/27/2009			Methods for Treating Neurofibromatosis	Owned by PTC.	
1300-219-888	United States	Docketed					Method for Treating Breast Cancer	Owned by PTC.	
1300-220-055	Singapore	Filed	200901748-4	3/15/2005			Carboline Derivatives Useful in the Inhibition of Anglogenesis\n	Owned by PTC.	
1400-201-228	Patent Cooperation Treaty	Filed	PCT/US2007/020462	9/21/2007			Pyrrolinone Compounds as Inhibitors of Bacterial Peptidyl tRNA Hydrolase and Uses Thereof	Owned by PTC.	
1400-201-999	United States	Filed	12/441,932	3/19/2009			Pyrrolinone Compounds as Inhibitors of Bacterial Peptidyl tRNA Hydrolase and Uses Thereof	Owned by PTC.	
1400-200-228	Patent Cooperation Treaty	Filed	PCT/US2007/020461	9/21/2007			Heretocyclic Inhibitors of Bacterial Peptidyl tRNA Hydrolase and Uses Thereof	Owned by PTC.	
1400-200-999	United States	Filed	12/441,929	3/19/2009			Heretocydic Inhibitors of Bacterial Peptidyl tRNA Hydrolase and Uses Thereof	Owned by PTC.	
1500-150-001	Canada	Filed	2578636.00	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	

1500-150-007	Australia	Filed	2005275182.00	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-008	New Zealand	Filed	553329.00	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-009	Mexico	Filed	MX/A/2007/000762	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-012	Japan	Filed	2007-522565	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-023	Hong Kong	Filed	7107221.90	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-056	India	Filed	542/DELNP/2007	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-146	China P.R.	Filed	200580031886.50	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-227	European Patent Convention	Filed	5773284.40	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-228	Patent Cooperation Treaty	Filed	PCT/US05/024882	7/14/2005			Thienopyridines for Treating Hepatitis C	Owned by PTC.	
1500-150-999	United States	Filed	11/180,779	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1500-165-001	Canada	Filed	2573185.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-007	Australia	Filed	2005275181.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-008	New Zealand	Filed	553173.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-009	Mexico	Filed	MX/A/2007/000481	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-012	Japan	Filed	2007-521619	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-015	Norway	Filed	20070194.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-023	Hong Kong	Filed	7107222.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-026	Colombia	Filed	7014310.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-037	Brazil	Filed	PI0511834	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-055	Singapore	Filed	200700197-7	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-056	India	Filed	595/DELNP/2007	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-068	Ecuador	Filed	SP-07-7252	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-076	Indonesia	Filed	W00200700138	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-117	Philippines	Filed	1-2007-500119	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-146	China P.R.	Filed	200580030803.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-147	South Africa	Filed	2007/01235	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-158	Israel	Filed	180645.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-200	Eurasian Patent Convention	Filed	200700243.00	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-227	European Patent Convention	Filed	5791638.90	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-228	Patent Cooperation Treaty	Filed	PCT/US05/024881	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-255	Ukraine	Filed	a22701232	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-461	Korea South	Filed	10-2007-7003515	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-165-999	United States	Filed	11/180,961	7/14/2005			Methods for Treating Hepatitis C	Owned by PTC.	
1500-168-333	United States	Filed	11/331,180	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-001	Canada	Filed	2636905.00	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-002	Argentina	Filed	P070100168	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-003	Chile	Filed	103-2007	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-004	Malaysia	Filed	20070066.00	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-005	Peru	Filed	40 2007	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-009	Mexico	Filed	MX/a/2008/009059	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-010	Thailand	Filed	701000139.00	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-011	Taiwan	Filed	96101523.00	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-012	Japan	Filed	2008-550442	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-013	Venezuela	Filed	2007-000066	1/13/2006			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-023	Hong Kong	Filed	9102637.60	3/19/2009			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-146	China P.R.	Filed	200780008902.80	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-227	European Patent Convention	Filed	7718004.00	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-228	Patent Cooperation Treaty	Filed	PCT/US07/00923	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-182-999	United States	Filed	11/653,448	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-001	Canada	Filed	2636916.00	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-002	Argentina	Filed	P080100139	1/11/2008			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-003	Chile	Filed	0078-2008	1/11/2008			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-005	Peru	Filed	121.00	1/11/2008			Methods for Treating Hepatitis C	Owned by PTC.	

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1500-183-009	Mexico	Filed	MX/2008/009059	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-010	Thailand	Filed	801000156.00	1/11/2008			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-011	Taiwan	Filed	97101218.00	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-012	Japan	Filed	2008-550455	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.	
1500-183-013	Venezuela	Filed	2008-000038	1/10/2008			Methods for Treating Hepatitis	Owned by PTC.	

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1500-183-016	Pakistan	Filed	17/2008	1/7/2008			Methods for Treating Hepatitis C	Owned by PTC.
1500-183-023	Hong Kong	Filed	9103304.60	4/7/2009			Methods for Treating Hepatitis C	Owned by PTC.
1500-183-146	China P.R.	Filed	200780008916.00	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.
1600-183-227	European Patent Convention	Filed	7716610.60	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.
1500-183-228	Patent Cooperation Treaty	Filed	PCT/US07/00996	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.
1500-183-333	United States	Filed	11/653,450	1/16/2007			Methods for Treating Hepatitis C	Owned by PTC.
1500-188-228	Patent Cooperation Treaty	Filed	PCT/US07/004721	2/23/2007			Combinations Comprising HCV Protease Inhibitor(s) and HCV IRES Inhibitor(s), and Methods of Treatmen	Owned by PTC.
1500-188-999	United States	Filed	12/281,022	2/23/2007			Combinations Comprising HCV Protease Inhibitor(s) and HCV IRES Inhibitor(s), and Methods of Treatmen	Owned by PTC.
1500-203-888	United States	Filed	61/166,893	4/6/2009			Methods for Treating Hepatitis C	Jointly owned under Research Collab with Schering-Plough (via its affiliate Essex Chemie). S-P has primary commercialization rights; PTC can conduct research and may gain commercialization rights under certain circumstances (e.g., reversion).
1500-208-888	United States	Filed	61/166,883	4/6/2009			Compounds and Methods for Antiviral Treatment	Jointly owned under Research Collab with Schering-Plough (via its affiliate Essex Chemie). S-P has primary commercialization rights; PTC can conduct research and may gain commercialization rights under certain circumstances (e.g., reversion).
1500-209-888	United States	Filed	61/166,913	4/6/2009			HCV Inhibitor and Therapeutic Agent Combinations	Jointly owned under Research Collab with Schering-Plough (via its affiliate Essex Chemie). S-P has primary commercialization rights; PTC can conduct research and may gain commercialization rights under certain circumstances (e.g., reversion).
1500-210-888	United States	Filed	61/166,922	4/6/2009			Indole Combinations	Jointly owned under Research Collab with Schering-Plough (via its affiliate Essex Chemie). S-P has primary commercialization rights; PTC can conduct research and may gain commercialization rights under certain circumstances (e.g.. reversion).
1500-211-888	United States	Filed	61/166,926	4/6/2009			HCV Inhibitor and Theurapeutic Agent Combinations	Jointly owned under Research Collab with Schering-Plough (via Its affiliate Essex Chemie). S-P has primary commercialization rights; PTC can conduct research and may gain commercialization rights under certain circumstances (e.g., reversion).
1000-100-777	United States	Granted	10/151,800	5/21/2002	6,583,309	6/24/2003	Ureas and Compositions thereof, and methods for their use for treating Cancer, inflammation or a Vir	Exclusively licensed to PTC pursuant to an agreement with the University of Medicine and Dentistry of New Jersey (“UMDNJ Agreement”).
1000-100-999	United States	Granted	09/679,728	10/4/2000	6.420,591	7/16/2002	Carbamates and Compositions thereof and Methods for their Use for Treating Cancer, Inflammation or a	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-101-333	United States	Filed	11/098,946	4/4/2005			Methods for identifying RNA Binding Compounds	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-101-666	United States	Granted	10/295,761	11/15/2002	6,875,736	4/5/2005	Methods for Identifying RNA Binding Compounds	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-101-999	United States	Granted	09/679,451	10/4/2000	6,503,713	1/7/2003	Methods for Identifying RNA Binding Compounds	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-124-666	United States	Filed	11/406,714	4/19/2006			A Method of Identifying an Antiviral Agent	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1000-124-698	Germany	Filed	854722	10/4/1996	69636675.4-08		Proteins Involved in Targeting of Peptidyl Transfer Center, and Corresponding Therapeutics Agents an	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-124-777	United States	Filed	09/625,790	7/26/2000			Proteins Involved in Targeting of Peptidyl Transfer Center, and Corresponding Therapeutics Agents an	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-124-999	United States	Filed	08/724,992	10/4/1996			Proteins Involved in Targeting of Peptidyl Transfer Center, and Corresponding Therapeutics Agents an	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-125-999	United States	Granted	08/888,865	7/7/1997	5,843,995	12/1/1998	Inhibition of HIV-1 Replication Using Ollgocarbamate Derivatives	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-001	Canada	Filed	2,329,267	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-007	Australia	Filed	43180/99	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-009	Mexico	Filed	11760	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-012	Japan	Filed	2000-550985	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-227	European Patent Convention	Granted	999533573	5/27/1999	7702838	7/5/2006	A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-461	Korea South	Filed	10-2000-7013415	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-698	Germany	Filed	1102838	5/27/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-129-777	United States	Granted	09/639,987	8/16/2000	6,486,305	11/26/2002	A Method of Modulating the Efficiency of Translation	Exclusively Bcensed to PTC pursuant to the UMDNJ	

							Termination and Degradation of Aberrant mRNA In	Agreement.
1000-129-999	United States	Filed	09/086,260	5/28/1999			A Method of Modulating the Efficiency of Translation Termination and Degradation of Aberrant mRNA In	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-130-666	United States	Granted	10/652,334	8/28/2003	6,989,256	1/24/2006	Subfamily of RNA Helicases Which are Modulators of the Fidelity of Translation Termination and Uses	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-130-777	United States	Granted	09/359,268	7/22/1999	6,630,294	10/7/2003	A Subfamily of RNA Helicases Which are Modulators of the Fidelity of Translation Termination and Use	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-130-999	United States	Filed	09/120,435	7/22/1999			A Subfamily of RNA Helicases Which are Modulators of the Fidelity of Translation Termination and Use	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-179-001	Canada	Filed	2,632,456	12/5/2005			A Novel HIV-1 Latency Model for High Throughput Screening	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.
1000-179-227	European Patent Convention	Filed	6844574.1	12/5/2005			A Novel HIV-1 Latency Model for High Throughput Screening	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.

Docket Number	Country	Status	Application Number	Application Date	Patent Number	Grant Date	Title (first 100 characters)	Ownership, Licensure and Disclosures	Additional Information
1000-179-228	Patent Cooperation Treaty	Filed	PCT/USG6/045483	12/5/2005			A Novel HIV-1 Latency Model for High Throughput Screening	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-179-999	United States	Filed	12/096,245	6/5/2008			A Novel HIV-1 Latency Model for High Throughput Screening	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-212-012	Japan	Filed	2009-089316	4/1/2009			Proteins involved in Targeting of Peptidyl Transfer Center, and Corresponding Therapeutics Agents an	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	
1000-212-227	European Patent Convention	Filed	9004759.8	3/31/2009			Methods for Identifying RNA Binding Compounds	Exclusively licensed to PTC pursuant to the UMDNJ Agreement.	

EXHIBIT A

The Collateral consists of all of Borrower's right, title and interest in and to the following personal property:

All goods, Accounts (including health-care receivables), Equipment, Inventory, raw materials, parts, supplies, packing and shipping materials, work in process, contract rights or rights to payment of money, leases, license agreements, franchise agreements, General Intangibles (except as provided below), commercial tort claims, documents, instruments (including any promissory notes), chattel paper (whether tangible or electronic), cash, deposit accounts, all certificates of deposit, fixtures, letters of credit rights (whether or not the letter of credit is evidenced by a writing), securities, and all other investment property, supporting obligations, and financial assets, whether now owned or hereafter acquired, wherever located; and

All Borrower's Books relating to the foregoing, and any and all claims, rights and interests in any of the above and all substitutions for, additions, attachments, accessories, accessions and improvements to and replacements, products, proceeds and insurance proceeds of any or all of the foregoing.

Notwithstanding the foregoing, the Collateral does not include (i) Borrower's Equipment constituting collateral for the Oxford Equipment Financing and the proceeds thereof (provided that upon payment in full of Borrower's Indebtedness under the Oxford Equipment Financing, such Equipment and the proceeds thereof shall automatically constitute, without further action required by any Person, Collateral), or (ii) any of the following, whether now owned or hereafter acquired except to the extent that it is necessary under applicable law to have a security interest in any of the following in order to have a perfected lien and security interest in and to the "IP Proceeds" defined below: any copyright rights, copyright applications, copyright registrations and like protections in each work of authorship and derivative work, whether published or unpublished; any patents, patent applications and like protections, including improvements, divisions, continuations, renewals, reissues, extensions, and continuations-in-part of the same; trademarks, trade names, service marks, mask works, rights of use of any name or domain names and, to the extent permitted under applicable law, any applications therefor, whether registered or not; and the goodwill of the business of Borrower connected with and symbolized thereby, know-how, operating manuals, trade secret rights, clinical and non-clinical data, rights to unpatented inventions; or (iii) exclusive and non-exclusive license agreements solely for the use of the intellectual property of a third party in which Borrower is licensee; provided, however, the Collateral shall include all Accounts, license and royalty fees and other revenues, proceeds, or income arising out of or relating to any of the property described in subparts (i), (ii), and (iii) preceding (other than sales proceeds of the Equipment securing the Oxford Equipment Financing until payment in full of Borrower's Indebtedness under the Oxford Equipment Financing) and any claims for damage by way of any past, present, or future infringement of any of the property described in subparts (ii) and (iii) preceding (collectively, the "**IP** Proceeds").

EXHIBIT B

Loan Payment/Advance Request Form

EXHIBIT C
COMPLIANCE CERTIFICATE

TO: Oxford Finance Corporation, as Collateral Agent Date:
FROM: PTC Therapeutics, Inc.

The undersigned authorized officer of PTC Therapeutics, Inc. ("Borrower") certifies, in the capacity as an officer of the Borrower, that under the terms and conditions of the Loan and Security Agreement between Borrower, Collateral Agent and the Lenders (the "Agreement"), (1) Borrower is in complete compliance for the period ending with all required covenants except as noted below, (2) there are no Events of Default, (3) all representations and warranties in the Agreement are true and correct in all material respects on this date except as noted below; provided, however, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; and provided, further that those representations and warranties expressly referring to a specific date shall be true, accurate and complete in all material respects as of such date, (4) Borrower, and each of its Subsidiaries, has timely filed all required tax returns and reports, and Borrower has timely paid all foreign, federal, state and local taxes, assessments, deposits and contributions owed by Borrower except as otherwise permitted pursuant to the terms of Section 5.8 of the Agreement, and (5) no Liens have been levied or claims made against Borrower or any of its Subsidiaries relating to unpaid employee payroll or benefits of which Borrower has not previously provided written notification to Collateral Agent. Attached are the required documents supporting the certification. The undersigned certifies, in the capacity as an officer of the Borrower, that these are prepared in accordance with GAAP consistently applied from one period to the next except as explained in an accompanying letter or footnotes. The undersigned acknowledges, in the capacity as an officer of Borrower, that no borrowings may be requested at any time or date of determination that Borrower is not in compliance with any of the terms of the Agreement, and that compliance is determined not just at the date this certificate is delivered. Capitalized terms used but not otherwise defined herein shall have the meanings given them in the Agreement.

Please indicate compliance status by circling Yes/No under "Complies" column.

Reporting Covenant	Required	Complies
Quarterly Financial Statements	Quarterly within 30 days	Yes o No o
Monthly Cash Certificate	Monthly within 5 Business Days	Yes o No o
Bank Statements	Monthly within 30 days	Yes o No o
Audited Financial Statements	Annually within 120 days after FYE	Yes o No o
Board Approved Operating and Capital Budgets	As approved by Board of Directors	Yes o No o
Compliance Certificate	Monthly within 30 days	Yes o No o

The following are the exceptions with respect to the certification above: (If no exceptions exist, state "No exceptions to note.")

By: _____
Name: _____
Title: _____

COLLATERAL AGENT USE ONLY

Received by: _____
AUTHORIZED SIGNER
Date: _____

Verified: _____
AUTHORIZED SIGNER
Date: _____

Compliance Status: Yes o No o

EXHIBIT D

SECURED PROMISSORY NOTE

$ Dated: September , 2009

FOR VALUE RECEIVED, the undersigned, PTC THERAPEUTICS, INC., a Delaware corporation ("Borrower") HEREBY PROMISES TO PAY to the order of [OXFORD/LENDER] ("Lender") the principal amount of Dollars ($) or such lesser amount as shall equal the outstanding principal balance of the Term Loan made to Borrower by Lender, plus interest on the aggregate unpaid principal amount of the Term Loan, at the rates and in accordance with the terms of the Loan and Security Agreement by and between Borrower and Oxford Finance Corporation, as Collateral Agent, and the Lenders (as amended, restated, supplemented or otherwise modified from time to time, the "Loan Agreement"). If not sooner paid, the entire principal amount and all accrued interest hereunder and under the Loan Agreement shall be due and payable on Maturity Date as set forth in the Loan Agreement.

Borrower agrees to pay any initial partial month interest payment from the date of this Secured Promissory Note (this "Note") to the first Payment Date ("Interim Interest") on the first Payment Date.

Principal, interest and all other amounts due with respect to the Term Loan, are payable in lawful money of the United States of America to Lender as set forth in the Loan Agreement and this Note. The principal amount of this Note and the interest rate applicable thereto, and all payments made with respect thereto, shall be recorded by Lender and, prior to any transfer hereof, endorsed on the grid attached hereto which is part of this Note.

The Loan Agreement, among other things, (a) provides for the making of a secured Term Loan to Borrower, and (b) contains provisions for acceleration of the maturity hereof upon the happening of certain stated events.

This Note may not be prepaid except as set forth in Section 2.2(c) and Section 2.2(d) of the Loan Agreement.

This Note and the obligation of Borrower to repay the unpaid principal amount of the Term Loan, interest on the Term Loan and all other amounts due Lender under the Loan Agreement is secured under the Loan Agreement.

Presentment for payment, demand, notice of protest and all other demands and notices of any kind in connection with the execution, delivery, performance and enforcement of this Note are hereby waived.

Borrower shall pay all reasonable fees and expenses, including, without limitation, reasonable attorneys' fees and costs, incurred by Lender in the enforcement or attempt to enforce any of Borrower's obligations hereunder not performed when due. This Note shall be governed by, and construed and interpreted in accordance with, the laws of the State of New York.

Note Register; Ownership of Note. The ownership of an interest in this Note shall be registered on a record of ownership maintained by Lender or its agent. Notwithstanding anything else in this Note to the contrary, the right to the principal of, and stated interest on, this Note may be transferred only if the transfer is registered on such record of ownership and the transferee is identified as the owner of an interest in the obligation. Borrower shall be entitled to treat the registered holder of this Note (as recorded on such record of ownership) as the owner in fact thereof for all purposes and shall not be bound to recognize any equitable or other claim to or interest in this Note on the part of any other person or entity.

IN WITNESS WHEREOF, Borrower has caused this Note to be duly executed by one of its officers thereunto duly authorized on the date hereof.

BORROWER:

PTC THERAPEUTICS, INC.

By _____
Name: _____
Title: _____

FIRST LOAN MODIFICATION AGREEMENT

This First Loan Modification Agreement (this "Loan Modification Agreement") is entered into as of March 26, 2010, by and among **(i) OXFORD FINANCE CORPORATION**, a Delaware corporation with an office located at 133 North Fairfax Street, Alexandria, Virginia 22314 ("**Oxford**"), as collateral agent ("**Collateral Agent**"), the Lenders listed on Schedule 1.1 of the Loan Agreement and otherwise party hereto from time to time (each a "**Lender**" and collectively, the "**Lenders**"), and **PTC THERAPEUTICS, INC**., a Delaware corporation ("**Borrower**").

DESCRIPTION OF EXISTING INDEBTEDNESS AND OBLIGATIONS. Among other indebtedness and obligations which may be owing by Borrower to the Lenders, Borrower is indebted to the Lenders pursuant to a loan arrangement dated as of September 21, 2009, evidenced by, among other documents (including without limitation, that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to Oxford in the original principal amount of $10,000,000, as amended, and that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to MidCap Financial, LLC in the original principal amount of $2,500,000, as amended), a certain Loan and Security Agreement dated as of September 21, 2009, among Borrower, Agent and the Lenders (as amended, the "Loan Agreement"). Capitalized terms used but not otherwise defined herein shall have the same meaning as in the Loan Agreement.

DESCRIPTION OF COLLATERAL. Repayment of the Obligations is secured by the Collateral as described in the Loan Agreement (together with any other document pursuant to which collateral security granted to Agent for the ratable benefit of the Lenders, the "Security Documents"). Hereinafter, the Security Documents, together with all other documents evidencing or securing the Obligations shall be referred to as the "Existing Loan Documents".

1. DESCRIPTION OF CHANGE IN TERMS.

Modifications to Loan Agreement.

1. The Loan Agreement shall be amended by adding the following to the definition of **Permitted Liens** appearing in Section 14.1 thereof:

"(1) Liens granted to American Express encumbering only a certificate of deposit in the amount of Two Hundred Thousand Dollars ($200,000.00) securing reimbursement obligations in connection with the Borrower's corporate credit cards."

2. The Loan Agreement shall be amended by adding the following to the definition of **Permitted Investments** appearing in Section 14.1 thereof:

"(f) Investments in connection with a certificate of deposit in the amount of Two Hundred Thousand Dollars ($200,000.00) pledged to American Express securing reimbursement obligations in connection with the Borrower's corporate credit cards."

3. The Loan Agreement shall be amended by inserting the following definitions appearing alphabetically in Section 14.1 thereof:

"**First Loan Modification Date**" is March 26, 2010."

2. EXPENSES. Borrower shall reimburse Agent and the Lenders for all reasonable legal fees and out-of pocket expenses incurred in connection with this Loan Modification Agreement.

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3. RATIFICATION OF LOAN DOCUMENTS. Borrower hereby ratifies, confirms, and reaffirms all terms and conditions of all security or other collateral granted to Agent for the ratable benefit of the Lenders, and confirms that the indebtedness secured thereby includes, without limitation, the Obligations.

4. NO DEFENSES OF BORROWER. Borrower hereby acknowledges and agrees that Borrower has no offsets, defenses, claims, or counterclaims against Agent and/or the Lenders with respect to the Obligations, or otherwise, and that if Borrower now has, or ever did have, any offsets, defenses, claims, or counterclaims against Agent and/or the Lenders, whether known or unknown, at law or in equity, all of them are hereby expressly WAIVED and Borrower hereby RELEASES Agent and/or the Lenders from any liability thereunder.

5. REPRESENTATIONS AND WARRANTIES. To induce Agent and Lenders to enter into this Loan Modification Agreement Borrower does hereby warrant, represent and covenant to Agent and Lenders that after giving effect to this Loan Modification Agreement (i) each representation or warranty of Borrower set forth in the Loan Agreement is hereby restated and reaffirmed as true and correct in all material respects on and as of the First Loan Modification Date as if such representation or warranty were made on and as of the First Loan Modification Date (except to the extent that any such representation or warranty expressly relates to a prior specific date or period), (ii) no Default or Event of Default has occurred and is continuing as of the date hereof and (iii) Borrower has the power and is duly authorized to enter into, deliver and perform this Loan Modification Agreement and this Loan Modification Agreement is the legal, valid and binding obligation of Borrower enforceable against Borrower in accordance with its terms.

6. CONTINUING VALIDITY. Except as expressly modified pursuant to this Loan Modification Agreement, the terms of the Existing Loan Documents remain unchanged and in full force and effect. The Lenders' agreement to modifications to the existing Obligations pursuant to this Loan Modification Agreement in no way shall obligate Agent or the Lenders to make any future modifications to the Obligations. Nothing in this Loan Modification Agreement shall constitute a satisfaction of the Obligations. It is the intention of Agent, the Lenders and Borrower to retain as liable parties all makers of Existing Loan Documents, unless the party is expressly released by the Lenders in writing. No maker will be released by virtue of this Loan Modification Agreement.

7. CONDITION PRECEDENT TO EFFECTIVENESS OF THIS LOAN MODIFICATION AGREEMENT. This Loan Modification Agreement shall become effective as of the First Loan Modification Date upon the receipt by Agent, in form and substance satisfactory to Agent and Lenders, of one or more counterparts of this Loan Modification Agreement duly executed and delivered by the Borrower, Agent and Lenders.

8. COUNTERPARTS. This Loan Modification Agreement may be executed in multiple counterparts, each of which shall be deemed to be an original and all of which when taken together shall constitute one and the same instrument.

9. GOVERNING LAW. THIS LOAN MODIFICATION AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE INTERNAL LAWS OF THE STATE OF NEW YORK APPLICABLE TO CONTRACTS MADE AND PERFORMED IN SUCH STATE WITHOUT REGARD TO THE PRINCIPLES THEREOF REGARDING CONFLICTS OF LAWS.

10. ENTIRE AGREEMENT. The Existing Loan Documents as and when amended through this Loan Modification Agreement embody the entire agreement between the parties hereto relating to the subject matter thereof and supersede all prior agreements, representations and understandings, if any, relating to the subject matter thereof.

[Remainder of Page Intentionally Left Blank –
Signature Page(s) to Follow.]

IN WITNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

BORROWER:

PTC THERAPEUTICS, INC.

By /s/ William Baird II
Name: William Baird
Title: CFO

LENDERS:

OXFORD FINANCE CORPORATION, as Agent and as a Lender

By /s/ John G. Henderson
Name: John G. Henderson
Title: Vice President & General Counsel

MIDCAP FUNDING III, LLC, as a Lender

By /s/ Luis Viera
Name: Luis Viera
Title: Managing Director

EXECUTION COPY

SECOND LOAN MODIFICATION AGREEMENT

This Second Loan Modification Agreement (this "Loan Modification Agreement") is entered into as of December 22, 2010, by and among (i) **OXFORD FINANCE CORPORATION**, a Delaware corporation with an office located at 133 North Fairfax Street, Alexandria, Virginia 22314 ("**Oxford**"), as collateral

agent ("**Collateral Agent**"), the Lenders identified on the signature pages hereto (each a "**Lender**" and collectively, the "**Lenders**"), and **PTC THERAPEUTICS, INC.**, a Delaware corporation ("**Borrower**")**.**

DESCRIPTION OF EXISTING INDEBTEDNESS AND OBLIGATIONS. Among other indebtedness and obligations which may be owing by Borrower to the Lenders, Borrower is indebted to the Lenders pursuant to a loan arrangement dated as of September 21, 2009, evidenced by, among other documents (including without limitation, that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to Oxford in the original principal amount of $10,000,000, as amended, and that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to MidCap Financial, LLC in the original principal amount of $2,500,000, as amended), and a certain Loan and Security Agreement dated as of September 21, 2009, among Borrower, Collateral Agent and the Lenders, as amended by a First Loan Modification Agreement dated as of March 26, 2010 among Borrower, Collateral Agent and the Lenders (as amended, the "Loan Agreement"). Capitalized terms used but not otherwise defined herein shall have the same meanings as in the Loan Agreement.

DESCRIPTION OF COLLATERAL. Repayment of the Obligations is secured by the Collateral as described in the Loan Agreement (together with any other document pursuant to which collateral security is granted to Collateral Agent for the ratable benefit of the Lenders, the "Security Documents"). Hereinafter, the Security Documents, together with all other documents evidencing or securing the Obligations shall be referred to as the "Existing Loan Documents".

1. DESCRIPTION OF CHANGE IN TERMS.

Modifications to Loan Agreement.

1. The Loan Agreement shall be amended by deleting the following text appearing as Section 2.2(a) thereof:

> "(a) Availability. Subject to the terms and conditions of this Agreement, the Lenders agree, severally and not jointly, (i) during the First Draw Period, to make term loans to Borrower in an aggregate amount up to Twelve Million Five Hundred Thousand Dollars ($12,500,000.00) according to each Lender's Term A Loan Commitment Percentage as set forth on Schedule 1.1 hereto (such term loans are hereinafter referred to singly as a "**Term A Loan**", and collectively as the "**Term A Loans**") and (ii) during the Second Draw Period, to make additional term loans to Borrower in an aggregate amount up to Twelve Million Five Hundred Thousand Dollars ($12,500,000.00) according to each Lender's Term B Loan Commitment Percentage as set forth on Schedule 1.1 hereto (subject to adjustment as provided on Schedule 1.1) (such term loans are hereinafter referred to singly as a "**Term B Loan**"**,** and collectively as the "**Term B Loans**"; each of the Term A Loans and the Term B Loans is referred to singly herein as a "**Term Loan**", and the Term A Loans and the Term B Loans are referred to collectively herein as the "**Term Loans**"). After repayment, no Term Loan may be re-borrowed."

and inserting in lieu thereof the following:

> "(a) Availability. Subject to the terms and conditions of this Agreement, the Lenders agree, severally and not jointly, (i) during the First Draw Period, to make term loans to Borrower in an aggregate amount up to Twelve Million Five Hundred Thousand Dollars ($12,500,000.00) according to each Lender's Term A Loan Commitment Percentage as set forth on Schedule 1.1 hereto (such term loans are hereinafter referred to singly as a "**Term A Loan**", and collectively as the "**Term A Loans**") and (ii) during the Second Draw Period, to make additional term loans to Borrower in an aggregate amount up to Ten Million Dollars ($10,000,000.00) according to each Lender's Term B Loan Commitment Percentage as set forth on Schedule 1.1 hereto (such term loans are hereinafter referred to singly as a "**Term B Loan**", and collectively as the "**Term B Loans**"; each of the Term A Loans and the Term B Loans is referred to singly herein as a "**Term Loan**", and the Term A Loans and the Term B Loans are referred to collectively herein as the "**Term Loans**"). After repayment, no Term Loan may be re-borrowed."

2. The Loan Agreement shall be amended by deleting the following text appearing as Section 2.5 (a) thereof:

> "(a) Facility Fee. A fully earned, non-refundable facility fee of Three Hundred Twelve Thousand Five Hundred Dollars ($312,500) to be shared between the Lenders pursuant to their respective Commitment Percentages. The facility fee shall be due and payable in two installments as follows: (i) $234,375 of the facility fee shall be due and payable on the Effective Date, and (ii) the remaining $78,125 of the facility fee shall be due and payable on the date on which Borrower achieves positive Phase IIb results for Borrower's Ataluren program (PTC124) for the Duchenne indication, which shall be evidenced by a final written determination to proceed, without further testing or trials, with the filing of a new drug application with the US Food and Drug Administration for Ataluren for the Duchenne indication, which such determination has been made by the joint development committee for Ataluren (which includes Genzyme, Inc. and Borrower) acting unanimously and supported by the findings of the independent safety monitoring board for the Phase IIb Ataluren clinical trial ("**Positive Pivotal Ataluren Data**"), whether or not Borrower draws down the Term B Loans. In the event Borrower does not achieve Positive Pivotal Ataluren Data on or prior to April 30, 2010, the second installment of the facility fee of $78,125 shall not be due and payable."

and inserting in lieu thereof the following:

> "(a) Facility Fee. A fully earned, non refundable facility fee of Two Hundred Eighty-One Thousand Two Hundred Fifty Dollars ($281,250), each installment of which is to be shared between the Lenders pursuant to their respective applicable Commitment Percentages. The facility fee shall be due and payable in two installments as follows: (i) $234,375 of the facility fee shall be due and payable on the Effective Date, and shall be shared between the Lenders based on their respective Term A Loan Commitment Percentages, and (ii) the remaining $46,875 of the facility fee shall be due and payable on the Second Loan Modification Date, whether or not Borrower draws

down the Term B Loans, and shall be shared between the Lenders based on their respective Term B Loan Commitment Percentages;"

3. The Loan Agreement shall be amended by deleting the date "December 31, 2010" appearing in Section 6.11 thereof, and replacing it with the date "March 31, 2011".

4. The Loan Agreement shall be amended by deleting the following definition appearing in Section 14.1 thereof:

""Second Draw Period" is the period commencing on the date on which Borrower achieves Positive Pivotal Ataluren Data and ending on April 30, 2010."

and inserting in lieu thereof the following:

""**Second Draw Period**" is the period commencing on the Second Loan Modification Date and ending on December 31, 2010."

5. The Loan Agreement shall be amended by inserting the following definition appearing alphabetically in Section 14.1 thereof:

" "**Second Loan Modification Date**" is December 22, 2010."

6. The Loan Agreement shall be amended by deleting Schedule 1.1 thereto in its entirety and replacing it with Schedule 1.1 attached to this Loan Modification as Exhibit A.

2. EXPENSES. Borrower shall reimburse Collateral Agent and the Lenders for all reasonable legal fees and out-of pocket expenses incurred in connection with this Loan Modification Agreement.

3. RATIFICATION OF LOAN DOCUMENTS. Borrower hereby ratifies, confirms, and reaffirms all terms and conditions of all security or other collateral granted to Collateral Agent for the ratable benefit of the Lenders, and confirms that the indebtedness secured thereby includes, without limitation, the Obligations.

4. PERFECTION CERTIFICATE. Borrower hereby ratifies, confirms and reaffirms, all and singular, the terms and disclosures contained in Borrower's Perfection Certificate dated as of September 21, 2009, as updated by Borrower's Perfection Certificate dated as of December 21, 2010, and acknowledges, confirms and agrees the disclosures and information Borrower provided to Collateral Agent and the Lenders in such Perfection Certificate, as updated, have not changed, as of the date hereof.

5. NO DEFENSES OF BORROWER. Borrower hereby acknowledges and agrees that Borrower has no offsets, defenses, claims, or counterclaims against Collateral Agent and/or the Lenders with respect to the Obligations, or otherwise, and that if Borrower now has, or ever did have, any offsets, defenses, claims, or counterclaims against Collateral Agent and/or the Lenders, whether known or unknown, at law or in equity, all of them are hereby expressly WAIVED and Borrower hereby RELEASES Collateral Agent and/or the Lenders from any liability thereunder.

6. REPRESENTATIONS AND WARRANTIES. To induce Collateral Agent and Lenders to enter into this Loan Modification Agreement Borrower does hereby warrant, represent and covenant to Collateral Agent and Lenders that after giving effect to this Loan Modification Agreement (i) each representation or warranty of Borrower set forth in the Loan Agreement is hereby restated and reaffirmed as true and correct in all material respects on and as of the Second Loan Modification Date as if such representation or warranty were made on and as of the Second Loan Modification Date (except to the extent that any such representation or warranty expressly relates to a prior specific date or period), (ii) no Default or Event of Default has occurred and is continuing as of the date hereof and (iii) Borrower has the power and is duly authorized to enter into, deliver and perform this Loan

Modification Agreement and this Loan Modification Agreement is the legal, valid and binding obligation of Borrower enforceable against Borrower in accordance with its terms.

7. CONTINUING VALIDITY. Except as expressly modified pursuant to this Loan Modification Agreement, the terms of the Existing Loan Documents remain unchanged and in full force and effect. The Lenders' agreement to modifications to the existing Obligations pursuant to this Loan Modification Agreement in no way shall obligate Collateral Agent or the Lenders to make any future modifications to the Obligations. Nothing in this Loan Modification Agreement shall constitute a satisfaction of the Obligations. It is the intention of Collateral Agent, the Lenders and Borrower to retain as liable parties all makers of Existing Loan Documents, unless the party is expressly released by the Lenders in writing. No maker will be released by virtue of this Loan Modification Agreement.

8. CONDITION PRECEDENT TO EFFECTIVENESS OF THIS LOAN MODIFICATION AGREEMENT. This Loan Modification Agreement shall become effective as of the Second Loan Modification Date upon the receipt by Collateral Agent, in form and substance satisfactory to Collateral Agent and Lenders, of one or more counterparts of this Loan Modification Agreement duly executed and delivered by the Borrower, Collateral Agent and Lenders.

9. COUNTERPARTS. This Loan Modification Agreement may be executed in multiple counterparts, each of which shall be deemed to be an original and all of which when taken together shall constitute one and the same instrument.

10. GOVERNING LAW. THIS LOAN MODIFICATION AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE INTERNAL LAWS OF THE STATE OF NEW YORK APPLICABLE TO CONTRACTS MADE AND PERFORMED IN SUCH STATE WITHOUT REGARD TO THE PRINCIPLES THEREOF REGARDING CONFLICTS OF LAWS.

11. ENTIRE AGREEMENT. The Existing Loan Documents as and when amended through this Loan Modification Agreement embody the entire agreement between the parties hereto relating to the subject matter thereof and supersede all prior agreements, representations and understandings, if any, relating to the subject matter thereof.

[Remainder of Page Intentionally Left Blank –
Signature Page(s) to Follow.]

IN WETNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

BORROWER:

PTC THERAPEUTICS, INC.

By /s/ Stuart Peltz
Name: Stuart Peltz
Title: President and Chief Executive Officer

LENDERS:

OXFORD FINANCE CORPORATION, as Collateral Agent and as a Lender

By /s/ Hans Houser
Name: Hans Houser
Title: Chief Credit Officer

MIDCAP FUNDING III, LLC (as assignee of MidCap Financial, LLC), as a Lender

By /s/ Luis Viera
Name: Luis Viera
Title: Managing Director

EXHIBIT A

SCHEDULE 1.1

LENDERS AND COMMITMENTS

Lender	Term A Loan Commitment	Commitment Percentage
Oxford Finance Corporation	$ 10,000,000	80%
Midcap Funding III, LLC	$ 2,500,000	20%
TOTAL	$ 12,500,000	100.00%

Lender	Term B Loan Commitment	Commitment Percentage
Oxford Finance Corporation	$ 5,000,000	50%
Midcap Funding III, LLC	$ 5,000,000	50%
TOTAL	$ 10,000,000	100.00%

Lender	Aggregate Commitments	Commitment Percentage
Oxford Finance Corporation	$ 15,000,000	66.67%
Midcap Funding III, LLC	$ 7,500,000	33.33%
TOTAL	$ 22,500,000	100.00%

EXECUTION VERSION

THIRD LOAN MODIFICATION AGREEMENT

This Third Loan Modification Agreement (this "Loan Modification Agreement") is entered into as of February 13, 2013, by and among **OXFORD FINANCE LLC,** a Delaware limited liability company (successor in interest to Oxford Finance Corporation, a Delaware corporation) with an office located at 133 North Fairfax Street, Alexandria, Virginia 22314 ("**Oxford**"), as collateral agent ("**Collateral Agent**"), the Lenders from time to time party to the Loan Agreement (each a "**Lender**" and collectively, the "**Lenders**"), and **PTC THERAPEUTICS, INC.,** a Delaware corporation ("**Borrower**").

1. DESCRIPTION OF EXISTING INDEBTEDNESS AND OBLIGATIONS. Among other indebtedness and obligations which may be owing by Borrower to the Lenders, Borrower is indebted to the Lenders pursuant a certain a loan arrangement dated as of September 21, 2009, evidenced by, among other documents (including without limitation, that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to Oxford in the original principal amount of $10,000,000, as amended, that certain Secured Promissory Note dated September 21, 2009 by Borrower payable to MidCap Financial, LLC in the original principal amount of $2,500,000, as amended, that certain Secured Promissory Note dated December 22, 2010 by Borrower payable to Oxford in the original principal amount of $5,000,000, as amended, and that certain Secured Promissory Note dated December 22, 2010 by Borrower payable to MidCap Funding III, LLC in the original principal amount of $5,000,000), a certain Loan and Security Agreement dated as of September 21, 2009, among Borrower, Collateral Agent and the Lenders, as amended by a First Loan Modification Agreement dated as of March 26, 2010 among Borrower, Collateral Agent and the Lenders, and as further amended by a Second Loan Modification Agreement dated as of December 22, 2010 among Borrower, Collateral Agent and the Lenders (as may be

amended, restated, or otherwise modified from time to time, the "**Loan Agreement**"). Capitalized terms used but not otherwise defined herein shall have the same meanings as in the Loan Agreement.

2. DESCRIPTION OF COLLATERAL. Repayment of the Obligations is secured by the Collateral as described in the Loan Agreement (together with any other document pursuant to which collateral security is granted to Collateral Agent for the ratable benefit of the Lenders, the "**Security Documents**"). Hereinafter, the Security Documents, together with all other documents evidencing or securing the Obligations shall be referred to as the "**Existing Loan Documents**".

3. DESCRIPTION OF CHANGE IN TERMS.

Modifications to Loan Agreement.

i. The Loan Agreement shall be amended by adding the following text at the end of Section 7.7 thereof:

"; provided, however, that, notwithstanding any provision in this Section 7.7 or elsewhere in Section 7, Borrower may establish, and make Investments from time to time in **PTC THERAPEUTICS LIMITED,** a wholly-owned subsidiary registered under the laws of England and Wales with registration number 07965055 and whose registered office is at 12 York Gate, London, United Kingdom, NW1, 4QS (the "**UK Subsidiary**") provided that (i) the UK Subsidiary shall execute and deliver to Collateral Agent a guaranty of the Obligations in form and substance satisfactory to the Lenders substantially the form attached as **Exhibit E** hereto and take all such action as may be reasonably required by Collateral Agent (as security trustee for the Finance Parties (as defined in the floating charge hereafter described)) to grant a continuing floating charge and security interest in and to the all of the assets of such Subsidiary (with such exclusions as shall be set forth in the definition of "Charged Assets" in such floating charge) under the laws of the United Kingdom England and Wales, and (B) Borrower shall execute and deliver to Collateral Agent (as security trustee for the Finance Parties (as defined in the Charge over Shares hereafter described)) a Charge over Shares in form and substance reasonably satisfactory to the Lenders and take all such action as may be reasonably required by Collateral Agent to grant a continuing fixed charge and security interest in and to the all of the stock, units or other evidence of ownership of the UK Subsidiary, (ii) at no time shall Borrower make any Investment in the UK Subsidiary other than cash reasonably necessary to (A) finance the creation of the UK Subsidiary and the payment of any franchise fees, taxes, fees of professional advisors (including but not limited to attorneys and accountants) and other costs incurred or reasonably expected to be incurred in the ordinary course of business to maintain the existence of the UK Subsidiary, and (B) finance any filing fees payable by the UK Subsidiary in connection with the filing by the UK Subsidiary of a Marketing Authorization Application and related filings in the European Union for Borrower's Ataluren program (the "**MAA**"), together with fees of professional advisors (including but not limited to attorneys and patent agents) and other costs incurred or reasonably expected to be incurred in the ordinary course of business to obtain and maintain marketing authorizations and related governmental approvals for Borrower's Ataluren program and to file, prosecute, maintain, defend and enforce intellectual property rights owned or controlled by the UK Subsidiary, (iii) at no time shall the UK Subsidiary incur indebtedness in excess of Twenty Five Thousand Dollars ($25,000) (or the equivalent amount in British £Pounds Sterling) in the aggregate or own any material assets other than its rights in respect of the MAA, any related rights to intellectual property licensed on a non-exclusive basis from Borrower, and any rights in intercompany agreements between the UK Subsidiary and Borrower (provided that any such non-exclusive licenses of rights in intellectual property, and any such intercompany agreements, shall be subject to the approval of the Lenders in their reasonable discretion and shall not transfer ownership of any intellectual property to the UK Subsidiary), (iv) notwithstanding the foregoing, in no event shall Borrower's aggregate Investments in the UK Subsidiary in any fiscal year exceed One Hundred Thousand Dollars ($100,000) (or the

equivalent amount in British £Pounds Sterling) or such greater amount as may approved in writing by the Required Lenders and (v) Borrower shall not transfer any Equipment to the UK Subsidiary, including, without limitation, the Equipment financed with the Oxford Equipment Financing ."

ii. The Loan Agreement shall be amended by inserting the following definitions appearing alphabetically in Section 14.1 thereof:

"**Third Loan Modification Date**" is February 13, 2013.

"**UK Subsidiary**" is defined in Section 7.7."

iii. The Loan Agreement shall be amended by amending and restating the definition of "Permitted Investments" in Section 14.1 thereof to read as follows:

"**Permitted Investments**" are:

(a) Investments shown on the Perfection Certificate and existing on the Effective Date; and

(b) Cash Equivalents;

(c) Investments consisting of the endorsement of negotiable instruments for deposit or collection or similar transactions in the ordinary course of Borrower;

(d) unsecured Investments to trade creditors incurred in the ordinary course of Borrower's business (e.g., up front payments under a vendor agreement);

(e) Investments in the UK Subsidiary provided such Investments are made in compliance with the proviso to Section 7.7; and

(f) Investments made pursuant to Borrower's Investment Policy Guidelines dated March 8, 2007, as in effect as of the Effective Date or as thereafter amended with the consent of the Required Lenders, which consent shall not be unreasonably withheld."

4. EXPENSES. Borrower shall reimburse Collateral Agent and the Lenders for all reasonable legal fees and out-of pocket expenses incurred in connection with this Loan Modification Agreement.

5. RATIFICATION OF LOAN DOCUMENTS. Borrower hereby ratifies, confirms, and reaffirms all terms and conditions of all security or other collateral granted to Collateral Agent for the ratable benefit of the Lenders, and confirms that the indebtedness secured thereby includes, without limitation, the Obligations.

6. PERFECTION CERTIFICATE. Borrower hereby ratifies, confirms and reaffirms, all and singular, the terms and disclosures contained in Borrower's Perfection Certificate dated as of September 21, 2009, as updated by Borrower's Perfection Certificate dated as of December 21, 2010, and as further updated by Borrower's Perfection Certificate dated as of February 13, 2013, and acknowledges, confirms and agrees the disclosures and information Borrower provided to Collateral Agent and the Lenders in such Perfection Certificate, as updated, have not changed, as of the date hereof.

7. NO DEFENSES OF BORROWER. Borrower hereby acknowledges and agrees that Borrower has no offsets, defenses, claims, or counterclaims against Collateral Agent and/or the Lenders with respect to the Obligations, or otherwise, and that if Borrower now has, or ever did have, any offsets, defenses, claims, or counterclaims against Collateral Agent and/or the Lenders, whether known or unknown, at law or in equity, all of them are hereby expressly WAIVED and Borrower hereby RELEASES Collateral Agent and/or the Lenders from any liability thereunder.

8. REPRESENTATIONS AND WARRANTIES. To induce Collateral Agent and Lenders to enter into this Loan Modification Agreement Borrower does hereby warrant, represent and covenant to Collateral Agent and Lenders that after giving effect to this Loan Modification Agreement (i) each representation or warranty of Borrower set forth in the Loan Agreement is hereby restated and reaffirmed as true and correct in all material respects on and as of the Second Loan Modification Date as if such representation or warranty were made on and as of the Second Loan Modification Date (except to the extent that any such representation or warranty expressly relates to a prior specific date or period), (ii) no Default or Event of Default has occurred and is continuing as of the date hereof and (iii) Borrower has the power and is duly authorized to enter into, deliver and perform this Loan Modification Agreement and this Loan Modification Agreement is the legal, valid and binding obligation of Borrower enforceable against Borrower in accordance with its terms.

9. CONTINUING VALIDITY. Except as expressly modified pursuant to this Loan Modification Agreement, the terms of the Existing Loan Documents remain unchanged and in full force and effect. The Lenders' agreement to modifications to the existing Obligations pursuant to this Loan Modification Agreement in no way shall obligate Collateral Agent or the Lenders to make any future modifications to the Obligations. Nothing in this Loan Modification Agreement shall constitute a satisfaction of the Obligations. It is the intention of Collateral Agent, the Lenders and Borrower to retain as liable parties all makers of Existing Loan Documents, unless the party is expressly released by the Lenders in writing. No maker will be released by virtue of this Loan Modification Agreement.

10. CONDITION PRECEDENT TO EFFECTIVENESS OF THIS LOAN MODIFICATION AGREEMENT. This Loan Modification Agreement shall become effective as of the Third Loan Modification Date upon the receipt by Collateral Agent, in form and substance satisfactory to Collateral Agent and Lenders, of one or more counterparts of this Loan Modification Agreement duly executed and delivered by the Borrower, Collateral Agent and Lenders.

11. COUNTERPARTS. This Loan Modification Agreement may be executed in multiple counterparts, each of which shall be deemed to be an original and all of which when taken together shall constitute one and the same instrument.

12. GOVERNING LAW. THIS LOAN MODIFICATION AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE INTERNAL LAWS OF THE STATE OF NEW YORK APPLICABLE TO CONTRACTS MADE AND PERFORMED IN SUCH STATE WITHOUT REGARD TO THE PRINCIPLES THEREOF REGARDING CONFLICTS OF LAWS.

13. ENTIRE AGREEMENT. The Existing Loan Documents as and when amended through this Loan Modification Agreement embody the entire agreement between the parties hereto relating to the subject matter thereof and supersede all prior agreements, representations and understandings, if any, relating to the subject matter thereof.

[Remainder of page intentionally left blank –
signature page follows]

IN WITNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

BORROWER:

PTC THERAPEUTICS, INC.

By: /s/ Mark E. Boulding
Name: Mark E. Boulding

Title: Executive Vice President
Chief Legal Officer

[Signatures continue on following page]

IN WITNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

COLLATERAL AGENT:

OXFORD FINANCE LLC

By: /s/ Mark Davis
Name: Mark Davis
Title: Vice President - Finance, Secretary & Treasurer

[Signatures continue on following page]

IN WITNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

LENDER:

OXFORD FINANCE FUNDING TRUST 2012-01

By: OXFORD FINANCE LLC, as servicer

By: /s/ Mark Davis
Name: Mark Davis
Title: Vice President - Finance, Secretary & Treasurer

[Signatures continue on following page]

IN WITNESS WHEREOF, the parties hereto have caused this Loan Modification Agreement to be executed as of the date first written above.

LENDER:

MIDCAP FUNDING III, LLC (as assignee of MidCap Financial, LLC)

By: /s/ Luis Viera
Name: Luis Viera
Title: Managing Director

Exhibit 21.1

Subsidiaries of PTC Therapeutics, Inc.

	Jurisdiction of Incorporation or Organization
PTC Therapeutics, Limited	England and Wales

WILMERHALE

+1 212 230 8800 (t)
+1 212 230 8888 (f)
wilmerhale.com

March 15, 2013

CONFIDENTIAL SUBMISSION

VIA EDGAR

U.S. Securities and Exchange Commission
Division of Corporate Finance
100 F Street, N.E.
Washington D.C. 20549

Re: Confidential Submission of Draft Form S-1 by PTC Therapeutics, Inc.

Ladies and Gentlemen:

On behalf of PTC Therapeutics, Inc. (the "Company"), we confidentially submit a draft Registration Statement on Form S-1 (the "Registration Statement") of the Company pursuant to Title I, Section 106 under the Jumpstart Our Business Startups Act of 2012 for non-public review by the Staff of the U.S. Securities and Exchange Commission (the "Staff") prior to the public filing of the Registration Statement.

Should members of the Staff have any questions or comments concerning this submission, please contact me at (212) 937-7334.

Sincerely,

/s/ Clark W. Petschek

Clark W. Petschek