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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

☑ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2023

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission file number: 001-35969

PTC Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
100 Corporate Court South Plainfield, NJ	**07080**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☑ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☑ No ☐

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

Large accelerated filer	☑	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☑

As of April 25, 2023, there were 74,190,700 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q include, among other things, statements about:

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;
- expectations with respect to our gene therapy platform, including our ability to commercialize Upstaza™ (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, in the European Economic Area, or EEA, any potential regulatory submissions and potential approvals for our product candidates, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;
- our ability to maintain our marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the EEA, which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;
- our ability to utilize results from Study 041 to support a conversion of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA to a standard marketing authorization and to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- the anticipated period of market exclusivity for Emflaza® (deflazacort) for the treatment of Duchenne muscular dystrophy in the United States under the Orphan Drug Act of 1983;
- our expectations with respect to the commercial status of Evrysdi® (risdiplam) and our program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc. and the Spinal Muscular Atrophy Foundation and our estimates regarding future revenues from sales-based royalty payments or the achievement of milestones in that program;
- our expectations and the potential financial impact and benefits related to our Collaboration and License Agreement with a subsidiary of Ionis Pharmaceuticals, Inc. including with respect to the timing of regulatory approval of Tegsedi® (inotersen) and Waylivra™ (volanesorsen) in countries in which we are licensed to commercialize them, the commercialization of Tegsedi and Waylivra, and our expectations with respect to royalty payments by us based on our potential achievement of certain net sales thresholds;
- the timing and scope of our commercialization of our products and product candidates;
- our estimates regarding the potential market opportunity for our products or product candidates, including the size of eligible patient populations and our ability to identify such patients;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for our products on adequate terms, or at all;

- our estimates regarding expenses, future revenues, third-party discounts and rebates, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies in our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;
- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;
- the ability and willingness of patients and healthcare professionals to access our products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the timing of, and our ability to obtain additional marketing authorizations for our products and product candidates;
- the ability of our products and our product candidates to meet existing or future regulatory standards;
- our ability to complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, according to the protocol agreed with the EMA;
- the potential receipt of revenues from future sales of our products or product candidates;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver our products and product candidates in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our expectations with respect to the COVID-19 pandemic and related response measures and their effects on our business, operations, clinical trials, potential regulatory submissions and approvals, our collaborators, contract research organizations, suppliers and manufacturers;
- our ability to complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- our expectations with respect to the potential financial impact and benefits of our leased biologics manufacturing facility and our ability to satisfy our obligations under the terms of the lease agreement for such facility;
- our ability to satisfy our obligations under the terms of the credit agreement with funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit and Wilmington Trust, National Association, as the administrative agent;

- our ability to satisfy our obligations under the indenture governing our 1.50% convertible senior notes due September 15, 2026;
- our regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, gene therapy, Bio-e, metabolic and oncology programs;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of our products and any product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; 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 Quarterly Report on Form 10-Q, particularly in Part II, Item 1A. Risk Factors as well as in Part I, Item 1A. Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2022, 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 Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2022 completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

In this Quarterly Report on Form 10-Q, unless otherwise stated or the context otherwise requires, references to “PTC,” “PTC Therapeutics,” “the Company,” “we,” “us,” “our,” and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

All website addresses given in this Quarterly Report on Form 10-Q are for information only and are not intended to be an active link or to incorporate any website information into this document.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.
Consolidated Balance Sheets (unaudited)
In thousands (except shares)

	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 167,495	$ 279,834
Marketable securities	118,808	130,871
Trade and royalty receivables, net	201,867	155,614
Inventory, net	26,649	21,808
Prepaid expenses and other current assets	98,573	105,658
Total current assets	613,392	693,785
Fixed assets, net	79,492	72,590
Intangible assets, net	686,205	705,891
Goodwill	82,341	82,341
Operating lease ROU assets	99,531	102,430
Deposits and other assets	47,878	48,582
Total assets	$ 1,608,839	$ 1,705,619
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 332,091	$ 320,366
Deferred revenue	214	1,351
Operating lease liabilities- current	9,598	9,370
Finance lease liabilities- current	1,857	3,000
Liability for sale of future royalties- current	97,874	72,149
Total current liabilities	441,634	406,236
Long-term debt	572,091	571,722
Contingent consideration payable	166,400	164,000
Deferred tax liability	102,831	102,834
Operating lease liabilities- noncurrent	100,442	100,860
Finance lease liabilities- noncurrent	17,184	18,675
Liability for sale of future royalties- noncurrent	665,677	685,737
Other long-term liabilities	141	2,641
Total liabilities	2,066,400	2,052,705
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 74,012,034 shares at March 31, 2023. Authorized 250,000,000 shares; issued and outstanding 73,104,692 shares at December 31, 2022.	73	72
Additional paid-in capital	2,339,886	2,305,020
Accumulated other comprehensive (loss) income	(1,587)	4,796
Accumulated deficit	(2,795,933)	(2,656,974)
Total stockholders' deficit	(457,561)	(347,086)
Total liabilities and stockholders' deficit	$ 1,608,839	$ 1,705,619

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Operations (unaudited)
In thousands (except shares and per share amounts)

	Three Months Ended March 31,	
	2023	2022
Revenues:		
Net product revenue	$ 187,557	$ 129,832
Collaboration revenue	6	7
Royalty revenue	30,831	18,896
Manufacturing revenue	1,988	—
Total revenues	220,382	148,735
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	14,144	10,135
Amortization of acquired intangible assets	39,415	23,473
Research and development	195,124	140,078
Selling, general and administrative	86,914	73,271
Change in the fair value of deferred and contingent consideration	2,400	(11,700)
Total operating expenses	337,997	235,257
Loss from operations	(117,615)	(86,522)
Interest expense, net	(27,331)	(23,514)
Other income (expense), net	9,956	(11,855)
Loss before income tax expense	(134,990)	(121,891)
Income tax expense	(3,969)	(4,835)
Net loss attributable to common stockholders	$ (138,959)	$ (126,726)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	73,729,284	71,215,105
Net loss per share—basic and diluted (in dollars per share)	$ (1.88)	$ (1.78)

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Comprehensive Loss (unaudited)
In thousands

	Three Months Ended March 31,	
	2023	2022
Net loss	$ (138,959)	$ (126,726)
Other comprehensive income (loss) :		
Unrealized gain (loss) on marketable securities, net of tax	54	(2,913)
Foreign currency translation (loss) gain, net of tax	(6,437)	8,587
Comprehensive loss	$ (145,342)	$ (121,052)

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Stockholders' (Deficit) Equity (unaudited)
In thousands (except shares)

Three months ended March 31, 2023	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2022	73,104,692	$ 72	$ 2,305,020	$ 4,796	$ (2,656,974)	$ (347,086)
Exercise of options	211,561	—	5,655	—	—	5,655
Restricted stock vesting and issuance, net	695,781	1	—	—	—	1
Share-based compensation expense	—	—	28,815	—	—	28,815
Receivable from investor	—	—	396	—	—	396
Net loss	—	—	—	—	(138,959)	(138,959)
Comprehensive loss	—	—	—	(6,383)	—	(6,383)
Balance, March 31, 2023	74,012,034	$ 73	$ 2,339,886	$ (1,587)	$ (2,795,933)	$ (457,561)

Three months ended March 31, 2022	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity (deficit)
	Shares	Amount				
Balance, December 31, 2021	70,828,226	$ 71	$ 2,123,606	$ (24,282)	$ (2,097,957)	$ 1,438
Exercise of options	97,188	—	2,444	—	—	2,444
Restricted stock vesting and issuance, net	411,627	—	—	—	—	—
Share-based compensation expense	—	—	26,589	—	—	26,589
Net loss	—	—	—	—	(126,726)	(126,726)
Comprehensive income	—	—	—	5,674	—	5,674
Balance, March 31, 2022	71,337,041	$ 71	$ 2,152,639	$ (18,608)	$ (2,224,683)	$ (90,581)

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Cash Flows (unaudited)
In thousands

	Three Months Ended March 31,	
	2023	2022
Cash flows from operating activities		
Net loss	$ (138,959)	$ (126,726)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	42,825	26,314
Non-cash operating lease expense	2,942	1,908
Non-cash royalty revenue related to sale of future royalties	(13,237)	(8,113)
Non-cash interest expense on liability related to sale of future royalties	18,902	18,874
Change in valuation of deferred and contingent consideration	2,400	(11,700)
Unrealized loss (gain) on ClearPoint Equity Investments	39	1,049
Unrealized (gain) loss on ClearPoint convertible debt security	(59)	1,542
Unrealized (gain) loss on marketable securities- equity investments	(2,166)	6,477
Disposal of asset	—	79
Amortization of (discounts) premiums on investments, net	(85)	887
Deferred income taxes	(4)	—
Amortization of debt issuance costs	438	464
Share-based compensation expense	28,815	26,589
Unrealized foreign currency transaction (gains) losses, net	(10,057)	2,135
Changes in operating assets and liabilities:		
Inventory, net	(4,612)	350
Prepaid expenses and other current assets	57,611	25,118
Trade and royalty receivables, net	(43,426)	(28,372)
Deposits and other assets	743	(510)
Accounts payable and accrued expenses	33,524	(30,680)
Other liabilities	(3,988)	(3,089)
Deferred revenue	(1,137)	—
Net cash used in operating activities	$ (29,491)	$ (97,404)
Cash flows from investing activities		
Purchases of fixed assets	$ (10,270)	$ (9,312)
Purchases of marketable securities- available for sale	—	(39,035)
Sale and redemption of marketable securities- available for sale	12,500	167,101
Sale and redemption of marketable securities- equity investments	2,196	2,423
Acquisition of product rights and licenses	(33,397)	(72,134)
Net cash (used in) provided by investing activities	$ (28,971)	$ 49,043
Cash flows from financing activities		
Proceeds from exercise of options	5,655	2,444
Debt issuance costs related to senior secured term loan	(182)	—
Payment of finance lease principal	(1,379)	(1,276)
Net cash provided by financing activities	$ 4,094	$ 1,168
Effect of exchange rate changes on cash	(7,963)	1,653
Net decrease in cash and cash equivalents	(62,331)	(45,540)
Cash and cash equivalents, and restricted cash beginning of period	295,925	197,218
Cash and cash equivalents, and restricted cash end of period	$ 233,594	$ 151,678
Supplemental disclosure of cash information		
Cash paid for interest	$ 12,956	$ 6,130
Cash paid for income taxes	2,215	1,987
Supplemental disclosure of non-cash investing and financing activity		
Unrealized gain (loss) on marketable securities, net of tax	$ 54	$ (2,913)
Right-of-use assets obtained in exchange for operating lease obligations	—	587
Acquisition of product rights and licenses	19,406	12,589
Debt issuance costs related to senior secured term loan	45	—
Capital expenditures unpaid at the end of period	28	—
Milestone payable	32,500	—

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
March 31, 2023
In thousands (except share and per share amounts unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes that this allows it to maximize value for all of its stakeholders.

PTC has a portfolio pipeline that includes several commercial products and product candidates in various stages of development, including clinical, pre-clinical and research and discovery stages, focused on the development of new treatments for multiple therapeutic areas for rare diseases relating to neurology, metabolism and oncology.

The Company has two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy ("DMD"), a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area (the "EEA") for the treatment of nonsense mutation Duchenne muscular dystrophy ("nmDMD") in ambulatory patients aged 2 years and older and in Russia for the treatment of nmDMD in patients aged two years and older. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency ("EMA") of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. The marketing authorization in the EEA was last renewed in June 2022 and is effective, unless extended, through August 5, 2023. In February 2023, the Company submitted a marketing authorization renewal request to the EMA. In September 2022, the Company submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. The Company expects an opinion from the Committee for Medicinal Products for Human Use in the second quarter of 2023.

Translarna is an investigational new drug in the United States. Following the Company's announcement of top-line results from the placebo-controlled trial of Study 041 in June 2022, the Company submitted a meeting request to the U.S. Food and Drug Administration ("FDA") to gain clarity on the regulatory pathway for a potential re-submission of a New Drug Application ("NDA") for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support an NDA re-submission. The Company then had an informal meeting with the FDA, during which the Company discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, the Company plans to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

The Company has a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system ("CNS") including Upstaza (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase ("AADC") deficiency ("AADC deficiency"), a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the European Commission approved

Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. The Company is also preparing and anticipates submitting a biologics license application ("BLA") to the FDA for Upstaza for the treatment of AADC deficiency in the United States. The Company is in the process of responding to the FDA's queries and this could result in a shift in timing of its expected BLA submission for Upstaza from the second quarter to the third quarter of 2023.

The Company holds the rights for the commercialization of Tegsedi® (inotersen) and Waylivra® (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to the Collaboration and License Agreement (the "Tegsedi-Waylivra Agreement"), dated August 1, 2018, by and between the Company and Akcea Therapeutics, Inc. ("Akcea"), a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, the European Union (the "EU") and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis ("hATTR amyloidosis"). The Company began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and it continues to make Tegsedi available in certain other countries within Latin America and the Caribbean through early access programs ("EAP Programs"). In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome ("FCS") in Brazil and the Company began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP Programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy ("FPL"). Waylivra has also received marketing authorization in the EU for the treatment of FCS.

The Company also has a spinal muscular atrophy ("SMA") collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc. (referred to collectively as "Roche") and the Spinal Muscular Atrophy Foundation ("SMA Foundation"). The SMA program has one approved product, Evrysdi® (risdiplam), which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA and the Company expects the EMA to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in 2023. In addition to the Company's SMA program, the Company's splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease ("HD"). The Company initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. The Company expects interim data from the initial 12-week portion of the Phase 2 study in the second quarter of 2023.

The Company's Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in the Company's Bio-e platform are vatiquinone and utreloxastat. The Company initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures in the third quarter of 2020. The Company has completed enrollment in this trial after previously experiencing delays in enrollment due to the COVID-19 pandemic. The Company anticipates results from the Phase 2/3 trial to be available in the second quarter of 2023. The Company also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipates results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, the Company completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. The Company initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

The most advanced molecule in the Company's metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. The Company initiated a registration-directed Phase 3 trial for sepiapterin for phenylketonuria ("PKU") in the third quarter of 2021 and expects results from Part 2 of this trial to be available in May 2023.

Unesbulin is the Company's most advanced oncology agent. The Company completed its Phase 1 trials evaluating unesbulin in leiomyosarcoma ("LMS") and diffuse intrinsic pontine glioma ("DIPG") in the fourth quarter of 2021. The Company initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. The Company expects to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG in the fourth quarter of 2023.

In addition, the Company has a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas for rare diseases.

As of March 31, 2023, the Company had an accumulated deficit of approximately $2,795.9 million. The Company has financed its operations to date primarily through the private offerings in September 2019 of 1.50% convertible senior notes due 2026 (see Note 9), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offering" of its common stock, its initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement dated as of July 17, 2020, by and among the Company, RPI 2019 Intermediate Finance Trust ("RPI"), and, solely for the limited purposes set forth therein, Royalty Pharma PLC (the "Royalty Purchase Agreement") (see Note 2), net proceeds from the Company's' borrowings under its credit agreement with Blackstone (see Note 9), private placements of its convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates. The Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. The Company has also relied on revenue associated with milestone and royalty payments from Roche pursuant to the License and Collaboration Agreement (the "SMA License Agreement") dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation, under its SMA program. The Company expects that cash flows from the sales of its products, milestone and royalty payments from Roche, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

2. Summary of significant accounting policies

The Company's complete listing of significant accounting policies is set forth in Note 2 of the notes to the Company's audited financial statements as of December 31, 2022 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 21, 2023 (the "2022 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2023 and for the three months ended March 31, 2023 and 2022 has been prepared by the Company, without audit, pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with generally accepted accounting principles in the United States ("GAAP") have been condensed or omitted pursuant to such rules and regulations. These interim financial statements should be read in conjunction with the Company's audited financial statements as of December 31, 2022 and notes thereto included in the 2022 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2023 and for the three months ended March 31, 2023 and 2022 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair

statement of financial position, results of operations, stockholders' (deficit) equity, and cash flows. The results of operations for the three months ended March 31, 2023 are not necessarily indicative of the results to be expected for the year ended December 31, 2023 or for any other interim period or for any other future year.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, royalty revenue, certain accruals related to the Company's research and development expenses, valuation procedures for liability for sale of future royalties, valuation procedures for convertible notes, fair value of the contingent consideration, and the provision for or benefit from income taxes. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

Restricted cash

Restricted cash included in deposits and other assets on the consolidated balance sheet contains an unconditional, irrevocable and transferable letter of credit that was entered into during the twelve-month period ended December 31, 2019 in connection with obligations under a facility lease for the Company's leased biologics manufacturing facility in Hopewell Township, New Jersey. The amount of the letter of credit is $7.5 million, is to be maintained for a term of not less than five years and has the potential to be reduced to $3.8 million if after five years the Company is not in default of its lease. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit that was entered into during June 2022 in connection with obligations for the Company's new facility lease in Warren, New Jersey. The amount of the letter of credit is $8.1 million and has the potential to be reduced to $4.1 million if after five years the Company is not in default of its lease. Both amounts are classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the letter of credit. Restricted cash also includes a bank guarantee of $0.5 million denominated in a foreign currency. Restricted cash also contains $50.0 million relating to funding the reserve account pursuant to the Blackstone Credit Agreement (as defined herein). This amount is included in prepaid and other current assets on the consolidated balance sheet. Refer to Note 9 for further details.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheet that sum to the total of the same amounts shown in the statement of cash flows:

	End of period- March 31, 2023	Beginning of period- December 31, 2022
Cash and cash equivalents	$ 167,495	$ 279,834
Restricted cash noncurrent included in deposits and other assets	16,099	16,091
Restricted cash current included in prepaid expenses and other current assets	50,000	—
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 233,594	$ 295,925

Marketable securities

The Company's marketable securities consists of both debt securities and equity investments. The Company considers its investments in debt securities with original maturities of greater than 90 days to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses within accumulated other comprehensive income. The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. If the criteria are not met, the Company evaluates whether the decline in fair value has resulted from a credit loss or other factors. In making this assessment, management considers, among other factors, the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating

agency, and adverse conditions specifically related to the security. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security are compared to the amortized cost basis of the security. If the present value of the cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss, limited by the amount that the fair value is less than the amortized costs basis. Any impairment that has not been recorded through an allowance for credit losses is recognized in other comprehensive income. For the three months ended March 31, 2023 and 2022, no allowance was recorded for credit losses.

Marketable securities that are equity investments are measured at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are components of other (expense) income, net within the consolidated statement of operations.

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Products which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense. Amounts related to clinical development programs and marketing efforts are immaterial.

The following table summarizes the components of the Company's inventory for the periods indicated:

	March 31, 2023	December 31, 2022
Raw materials	$ 1,097	$ 1,078
Work in progress	18,438	14,074
Finished goods	7,114	6,656
Total inventory	$ 26,649	$ 21,808

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. For the three months ended March 31, 2023 and 2022, the Company recorded inventory write-downs of $0.1 million and $0.6 million, respectively, primarily related to product approaching expiration. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales. For the three months ended March 31, 2023 and 2022, these amounts were immaterial.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, storage costs, amortization of the acquired intangible asset, royalty payments associated with net product sales, and royalty payments to collaborative partners associated with royalty revenues and collaboration revenue related to milestones. Production costs are expensed as cost of product sales when the related products are sold or royalty revenues and collaboration revenue milestones are earned.

Revenue recognition

Net product revenue

The Company's net product revenue primarily consists of sales of Translarna in territories outside of the U.S. for the treatment of nmDMD and sales of Emflaza in the U.S. for the treatment of DMD. The Company recognizes revenue when

its performance obligations with its customers have been satisfied. The Company's performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when the Company's customer obtains control of the product, which is typically upon delivery. The Company invoices its customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of the invoice date. The Company determines the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods the Company has yet to provide. As the Company has identified only one distinct performance obligation, the transaction price is allocated entirely to product sales. In determining the transaction price, a significant financing component does not exist since the timing from when the Company delivers product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

The Company records product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. The Company uses the expected value or most likely amount method when estimating its variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

For the three months ended March 31, 2023 and 2022, net product sales outside of the United States were $133.0 million and $81.2 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $115.1 million and $79.2 million of the net product sales outside of the United States for the three months ended March 31, 2023 and 2022, respectively. For the three months ended March 31, 2023 and 2022, net product sales in the United States were $54.6 million and $48.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $54.6 million, $44.6 million, and $25.9 million of net product sales, respectively. During the three months ended March 31, 2022, two countries, the United States and Brazil, accounted for at least 10% of the Company's net product sales, representing $48.6 million and $25.8 million of net product sales, respectively.

In relation to customer contracts, the Company incurs costs to fulfill a contract but does not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. The Company considers any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration and royalty revenue

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.

At the inception of a collaboration arrangement, the Company needs to first evaluate if the arrangement meets the criteria in Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 808 "Collaborative Arrangements" to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, the Company assesses the promises in the arrangement to identify distinct performance obligations.

For licenses of intellectual property, the Company assesses, at contract inception, whether the intellectual property is distinct from other performance obligations identified in the arrangement. If the licensing of intellectual property is determined to be distinct, revenue is recognized for nonrefundable, upfront license fees when the license is transferred to the customer and the customer can use and benefit from the license. If the licensing of intellectual property is determined

not to be distinct, then the license will be bundled with other promises in the arrangement into one distinct performance obligation. The Company needs to determine if the bundled performance obligation is satisfied over time or at a point in time. If the Company concludes that the nonrefundable, upfront license fees will be recognized over time, the Company will need to assess the appropriate method of measuring proportional performance.

For milestone payments, the Company assesses, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, the Company will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within the Company's control. If it is probable that a significant revenue reversal will not occur, the Company will estimate the milestone payments using the most likely amount method. The Company will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement. The Company recognizes royalties from product sales at the later of when the related sales occur or when the performance obligation to which the royalty has been allocated has been satisfied. If it is probable that a significant revenue reversal will not occur, the Company will estimate the royalty payments using the most likely amount method.

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.

For the three months ended March 31, 2023 and 2022, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial.

For the three months ended March 31, 2023 and 2022, the Company has recognized $30.8 million and $18.9 million of royalty revenue, respectively, related to Evrysdi.

Manufacturing Revenue

The Company has manufacturing services related to the production of plasmid deoxyribonucleic acid ("DNA") and adeno-associated virus ("AAV") vectors for gene therapy applications for external customers. Performance obligations vary but may include manufacturing plasmid DNA and/or AAV vectors, material testing, stability studies, and other services related to material development. The transaction prices for these arrangements are fixed and include amounts stated in the contracts for each promised service. Typically, the performance obligations within a manufacturing contract are highly interdependent, in which case, the Company will combine them into a single performance obligation. The Company has determined that the assets created have no alternative use to the Company, and the Company has an enforceable right to payment for the performance completed to date, therefore revenue related to these services are recognized over time and is measured using an output method based on performance of manufacturing milestones completed to date.

Manufacturing service contracts may also include performance obligations related to project management services or obtaining materials from third parties. The Company has determined that these are separate performance obligations for which revenue is recognized at the point in time the services are performed. For performance obligations related to obtaining third party materials, the Company has determined that it is the principal as the Company has control of the materials and has discretion in setting the price. Therefore, the Company recognizes revenue on a gross basis related to obtaining third party materials.

Certain arrangements require a portion of the contract consideration to be received in advance at the commencement of the contract, and such advance payment is initially recorded as a contract liability. A contract asset may be recognized in the event the Company's satisfaction of performance obligations outpaces customer billings.

For the three months ended March 31, 2023, the Company recognized $2.0 million of manufacturing revenue related to plasmid DNA and AAV vector production for external customers. No manufacturing revenue was recognized for the three months ended March 31, 2022. As of March 31, 2023 and December 31, 2022, the aggregate amount of transaction price

allocated to remaining performance obligations related to plasmid DNA and AAV vector production for external customers is $0.2 million and $1.4 million, respectively.

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer's financial condition and current economic trends. The Company also assesses whether an allowance for expected credit losses may be required which includes a review of the Company's receivables portfolio, which are pooled on a customer basis or country basis. In making its assessment of whether an allowance for credit losses is required, the Company considers its historical experience with customers, current balances, levels of delinquency, regulatory and legal environments, and other relevant current and future forecasted economic conditions. For the three months ended March 31, 2023 and 2022, no allowance was recorded for credit losses. The allowance for doubtful accounts was $0.4 million as of March 31, 2023 and $0.3 million as of December 31, 2022. Bad debt expense was immaterial for the three months ended March 31, 2023 and 2022.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the "Assigned Royalty Payment") of the Company's right to receive sales-based royalty payments (the "Royalty") on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement. In consideration for the sale of the Assigned Royalty Payments, RPI paid the Company $650.0 million in cash consideration. The Company has retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement, which remaining milestone payments equal $250.0 million in the aggregate as of March 31, 2023. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

The cash consideration obtained pursuant to the Royalty Purchase Agreement is classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is being amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. Refer to Note 9 for further details.

Indefinite-lived intangible assets

Indefinite-lived intangible assets consist of in process research and development ("IPR&D"). IPR&D acquired directly in a transaction other than a business combination is capitalized if the projects will be further developed or have an alternative future use; otherwise they are expensed. The fair values of IPR&D projects and license agreement assets acquired in business combinations are capitalized. Several methods may be used to determine the estimated fair value of the IPR&D and license agreement asset acquired in a business combination. The Company utilizes the "income method" and uses estimated future net cash flows that are derived from projected sales revenues and estimated costs. These projections are based on factors such as relevant market size, patent protection, and expected pricing and industry trends. The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate. Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset's fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived

intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. The Company considers many factors in evaluating whether the value of its intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, the Company's outlook and market performance of the Company's industry and recent and forecasted financial performance.

Goodwill

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company's business acquisitions accounted for using the acquisition method of accounting. Goodwill is not amortized and is subject to impairment testing at a reporting unit level on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired. The Company reassess its reporting units as part of its annual segment review. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount.

Income Taxes

The Organization for Economic Co-operation and Development ("OECD"), the European Community ("the EC"), and individual taxing jurisdictions where the Company and its affiliates do business have recently focused on issues related to the taxation of multinational corporations. The OECD has released its comprehensive plan to create an agreed set of international rules for fighting base erosion and profit shifting. In addition, the OECD, the EC and individual taxing jurisdictions are examining changes to how taxing rights should be allocated among countries considering the digital economy. As a result, the tax laws in the U.S. and other countries in which the Company and its affiliates do business could change on a prospective or retroactive basis and any such changes could materially adversely affect the Company's business.

On December 22, 2017, the U.S. government enacted the 2017 Tax Act, which significantly revised U.S. tax law by, among other provisions, lowering the U.S. federal statutory corporate income tax rate to 21%, imposing a mandatory one-time transition tax on previously deferred foreign earnings, and eliminating or reducing certain income tax deductions. The Global Intangible Low-Taxed Income ("GILTI") provisions of the 2017 Tax Act require the Company to include in its U.S. income tax return foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary's tangible assets. The Company has elected to account for GILTI tax in the period in which it is incurred, and therefore has not provided any deferred tax impacts of GILTI in its consolidated financial statements for the period ended March 31, 2023.

Starting in 2022, TCJA amendments to IRC Section 174 no longer permits an immediate deduction for research and development (R&D) expenditures in the tax year that such costs are incurred. Instead, these IRC Section 174 development costs must now be capitalized and amortized over either a five- or 15-year period, depending on the location of the activities performed. The new amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1, and runs until the midpoint of year five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil. As a result of this tax law change, the Company recorded a federal and state tax provision for the three months ended March 31, 2023, in the amount of $0.7 million and $2.9 million, respectively.

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.

On August 23, 2018, the Company completed its acquisition of Agilis Biotherapeutics, Inc. (“Agilis”), pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018 (the “Agilis Merger Agreement”), by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and the Company’s wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, (the “Agilis Merger”). The Company recorded a deferred tax liability in conjunction with the Agilis Merger of $122.0 million in 2018, related to the tax basis difference in the IPRD indefinite-lived intangibles acquired. The Company’s policy is to record a deferred tax liability related to acquired IPR&D which may eventually be realized either upon amortization of the asset when the research is completed, and a product is successfully launched or the write-off of the asset if it is abandoned or unsuccessful. In July 2022, the Company received EMEA approval for a portion of the IPR&D assets, and thus, began the amortization of the intangible.

Leases

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all leases. Operating and finance leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating and finance lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

Variable lease payments associated with the Company’s leases are recognized when the event, activity, or circumstance in the lease agreement on which those payments are assessed occurs. Variable lease payments are presented in the Company’s consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases.

Leases with an initial term of 12 months or less are not recorded on the consolidated balance sheet and the Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

A lessee is required to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company’s leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the commencement date in determining the present value of lease payments. The Company gives consideration to its recent debt issuances as well as publicly available data for instruments with similar characteristics when calculating its incremental borrowing rates.

The lease term for all of the Company’s leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor. Leasehold improvements are capitalized and depreciated over the lesser of useful life or lease term. See Note 3 Leases for additional information.

3. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under two noncancelable operating leases through August 2024, in addition to office and laboratory space in Bridgewater, New Jersey and other locations throughout the United States and office space in various countries for international employees primarily through workspace providers.

The Company also leases approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey pursuant to a Lease Agreement (the “Hopewell Lease”) with Hopewell Campus Owner LLC. The rental term of the Hopewell Lease commenced on July 1, 2020 and has an initial term of fifteen

years (the “Hopewell Initial Term”), with two consecutive ten year renewal periods, each at the Company’s option. The aggregate rent for the Hopewell Initial Term will be approximately $111.5 million. The rental rate for the renewal periods will be 95% of the Prevailing Market Rate (as defined in the Hopewell Lease) and determined at the time of the exercise of the renewal. The Company is also responsible for maintaining certain insurance and the payment of proportional taxes, utilities and common area operating expenses. The Hopewell Lease contains customary events of default, representations, warranties and covenants.

In May 2022, the Company entered into a Lease Agreement (the “Warren Lease”) with Warren CC Acquisitions, LLC (the “Warren Landlord”) relating to the lease of two entire buildings comprised of approximately 360,000 square feet of shell condition, modifiable space (the “Warren Premises”) at a facility located in Warren, New Jersey. The rental term of the Warren Lease commenced on June 1, 2022, with an initial term of seventeen years (the “Warren Initial Term”), followed by three consecutive five-year renewal periods at the Company’s option. The aggregate base rent for the Warren Initial Term will be approximately $163.0 million; provided, however, that if the Company is not subject to an Event of Default (as defined in the Warren Lease), the Company will be entitled to a base rent abatement over the first three years of the Warren Initial Term of approximately $18.6 million, reducing the Company’s total base rent obligation to $144.4 million. The rental rate for the renewal periods will be at the Fair Market Rental Value (as defined in the Warren Lease) and determined at the time of the exercise of the renewal. Beginning in the second lease year, the Company is also responsible for the payment of all taxes and operating expenses for the Warren Premises. As a result, the Company recorded an operating lease ROU asset of $28.9 million and an operating lease ROU liability of $28.9 million as of the commencement date.

The Company plans on developing the Warren Premises into office and laboratory space. The Company is entitled to an allowance of approximately $36.2 million to be provided by the Warren Landlord to be used towards such improvements. The Landlord is providing the allowance to cover those assets that are real property improvements, such as structural components, roofs, flooring, etc., whose useful lives are typically longer in nature. The Company evaluated the leasehold improvements under ASC 842 and determined that the Company will be the owner of the improvements, and therefore the $36.2 million allowance and $5.0 million due from the Landlord were treated as lease incentives at the commencement of the lease and included in the calculation of the lease ROU asset and lease ROU liability, effectively reducing both at Commencement Date. In connection with the execution of the Warren Lease, the Company also committed to fund a construction account with $3.6 million to go towards the Company’s improvements of the Warren Premises. Subject to the terms of the Warren Lease, the Company has a right of first offer to purchase the Warren Premises if the Warren Landlord receives a bona fide third party offer to purchase the Warren Premises or the Warren Landlord decides to sell the Warren Premises

On June 19, 2020, the Company entered into a commercial manufacturing service agreement for a term of 12.5 years with MassBiologics of the University of Massachusetts Medical School ("MassBio"). The Company determined that the agreement was a finance lease, for which the Company recorded a finance lease ROU asset for $41.4 million and corresponding finance lease liability for $41.4 million at the onset of the lease agreement. Given that the leased asset is designed for the production of PTC’s AADC program and would not have an alternate use outside the PTC gene therapy platform without incurring significant costs, the Company determined that the lease should be treated as research and development expense under ASC 730. Accordingly, the full $41.4 million relating to the finance lease ROU asset was written off and expensed to research and development during the year ended December 31, 2020. As of March 31, 2023, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent are $1.8 million and $17.2 million, respectively, and are directly related to the Company’s MassBio agreement. As of December 31, 2022, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent were $3.0 million and $18.7 million, respectively. Additionally, the Company recorded finance lease costs of $0.4 million and $0.4 million related to interest on the lease liability during the three months ended March 31, 2023 and 2022, respectively.

The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company’s leases have remaining operating lease terms ranging from 0.9 years to 16.2 years and certain of the leases include renewal options to extend the lease for up to 20 years. Rent expense was $7.1 million and $5.3 million for the three months ended March 31, 2023 and 2022, respectively.

The components of operating lease expense were as follows:

	Three Months Ended March 31, 2023	Three Months Ended March 31, 2022
Operating Lease Cost		
Fixed lease cost	$ 5,473	$ 4,126
Variable lease cost	1,353	1,076
Short-term lease cost	303	74
Total operating lease cost	$ 7,129	$ 5,276

Total operating lease cost is a component of operating expenses on the consolidated statements of operations.

Supplemental lease term and discount rate information related to leases was as follows as March 31, 2023 and December 31, 2022:

	March 31, 2023	December 31, 2022
Weighted-average remaining lease terms - operating leases (years)	11.57	11.61
Weighted-average discount rate - operating leases	8.63 %	8.61 %
Weighted-average remaining lease terms - finance lease (years)	9.76	10.01
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of March 31, 2023 and 2022:

	Three Months Ended March 31, 2023	2022
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,811	$ 3,411
Financing cash flows from finance lease	1,379	1,276
Operating cash flows from finance leases	1,621	1,724
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ —	$ 587

Future minimum lease payments under non-cancelable leases as of March 31, 2023 were as follows:

	Operating Leases	Finance Lease
2023 (excludes the three months ended March 31, 2023)	$ 11,575	$ —
2024	18,465	3,000
2025	20,434	3,000
2026	19,986	3,000
2027 and thereafter	193,792	18,000
Total lease payments	264,252	27,000
Less: Imputed Interest expense	154,212	7,959
Total	$ 110,040	$ 19,041

4. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provide 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 establish 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 marketable securities are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments.

The Company owns common stock in ClearPoint Neuro, Inc. ("ClearPoint") (formerly MRI Interventions, Inc.), a publicly traded medical device company. The ClearPoint equity investments (collectively, the "ClearPoint Equity Investments") represent financial instruments, and therefore, are recorded at fair value, which is readily determinable. The ClearPoint Equity Investments are components of deposits and other assets on the consolidated balance sheet. During the three months ended March 31, 2023 and 2022, the Company recorded unrealized losses of $0.1 million and $1.0 million, respectively. These unrealized losses are components of other income (expense), net within the consolidated statement of operations. The fair value of the ClearPoint Equity Investments was $10.9 million and $11.0 million as of March 31, 2023 and December 31, 2022, respectively. The Company classifies the ClearPoint Equity Investments as Level 1 assets within the fair value hierarchy, as the value is based on a quoted market price in an active market, which is not adjusted.

In January 2020, the Company purchased a $10.0 million convertible note from ClearPoint that the Company can convert into ClearPoint shares at a conversion rate of $6.00 per share at any point throughout the term of the loan, which matures five years from the purchase date. The Company determined that the convertible note represents an available for sale debt security and the Company has elected to record it at fair value under ASC 825. The Company classifies its ClearPoint convertible debt security as a Level 2 asset within the fair value hierarchy, as the value is based on inputs other than quoted prices that are observable. The fair value of the ClearPoint convertible debt security is determined at each reporting period by utilizing a Black-Scholes option pricing model, as well as a present value of expected cash flows from the debt security utilizing the risk free rate and the estimated credit spread as of the valuation date as the discount rate. During the three months ended March 31, 2023 and 2022, the Company recorded unrealized gains of $0.1 million and unrealized losses of $1.5 million, respectively. These unrealized gains and losses are components of other income (expense), net within the consolidated statement of operations. The fair value of the convertible debt security was $15.3 million and $15.2 million as of March 31, 2023 and December 31, 2022, respectively. The convertible debt security is considered to be long term and is included as a component of deposits and other assets on the consolidated balance sheet. Other than the ClearPoint Equity Investments and the ClearPoint convertible debt security, no other items included in deposits and other assets on the consolidated balance sheets are fair valued.

The Company has investments in mutual funds, including one that is denominated in a foreign currency. All of these are equity investments and are classified as marketable securities on the Company's consolidated balance sheets. These equity investments are reported at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are included as components of other income (expense), net within the consolidated statement of operations. For the three months ended March 31, 2023 and 2022, the Company had unrealized gains of $2.2 million and unrealized losses of $6.5 million relating to the equity investments still held at the reporting date, respectively. For the three months ended March 31, 2023 and 2022, the Company had redemptions of $2.2

million and $2.4 million, respectively. For the three months ended March 31, 2023 and 2022, the Company had foreign currency unrealized gains of $0.3 million and $0.7 million, respectively, relating to these equity investments.

Fair value of marketable securities that are classified as available for sale debt securities is based upon market prices using quoted prices in active markets for identical assets quoted on the last day of the period. In establishing the estimated fair value of the remaining available for sale debt securities, the Company used the fair value as determined by its investment advisors using observable inputs other than quoted prices.

The following represents the fair value using the hierarchy described above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of March 31, 2023 and December 31, 2022:

	March 31, 2023			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 10,249	$ —	$ 10,249	$ —
Marketable securities - equity investments	$ 108,559	$ 108,559	$ —	$ —
ClearPoint Equity Investments	$ 10,926	$ 10,926	$ —	$ —
ClearPoint convertible debt security	$ 15,290	$ —	$ 15,290	$ —
Contingent consideration payable- development and regulatory milestones	$ 85,500	$ —	$ —	$ 85,500
Contingent consideration payable- net sales milestones and royalties	$ 80,900	$ —	$ —	$ 80,900

	December 31, 2022			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 22,610	$ —	$ 22,610	$ —
Marketable securities - equity investments	$ 108,261	$ 108,261	$ —	$ —
ClearPoint Equity Investments	$ 10,965	$ 10,965	$ —	$ —
ClearPoint convertible debt security	$ 15,231	$ —	$ 15,231	$ —
Contingent consideration payable- development and regulatory milestones	$ 82,500	$ —	$ —	$ 82,500
Contingent consideration payable- net sales milestones and royalties	$ 81,500	$ —	$ —	$ 81,500

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended March 31, 2023 and December 31, 2022.

The following is a summary of marketable securities accounted for as available for sale debt securities at March 31, 2023 and December 31, 2022:

	March 31, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Corporate debt securities	$ 10,689	$ —	$ (440)	$ 10,249
Total	$ 10,689	$ —	$ (440)	$ 10,249

	December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 12,419	$ 5	$ —	$ 12,424
Corporate debt securities	10,685	—	(499)	10,186
Total	$ 23,104	$ 5	$ (499)	$ 22,610

For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. For the three months ended March 31, 2023 and 2022, no write downs occurred. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis, which may be maturity. The Company also reviews its available for sale debt securities in an unrealized loss position and evaluates whether the decline in fair value has resulted from credit losses or other factors. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may be related to credit issues. For the three months ended March 31, 2023 and 2022, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity.

For the three months ended March 31, 2023, the Company did not have any realized gains or losses from the sale of available for sale debt securities. For the three months ended March 31, 2022, the Company had $0.1 million realized losses from the sale of available for sale debt securities. Realized gains and losses are reported as a component of interest expense, net in the consolidated statement of operations. Reclassified amounts from other comprehensive items were determined using the actual realized gains and losses from the sales of marketable securities.

The unrealized losses and fair values of available for sale debt securities that have been in an unrealized loss position for a period of less than and greater than or equal to 12 months as of March 31, 2023 are as follows:

	March 31, 2023					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ —	—	(440)	10,249	(440)	$ 10,249
Total	$ —	$ —	$ (440)	$ 10,249	$ (440)	$ 10,249

The unrealized losses and fair values of available for sale debt securities that have been in an unrealized loss position for a period of less than and greater than or equal to 12 months as of December 31, 2022 are as follows:

	December 31, 2022					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ —	—	(499)	10,186	(499)	$ 10,186
Total	$ —	$ —	$ (499)	$ 10,186	$ (499)	$ 10,186

Available for sale debt securities at March 31, 2023 and December 31, 2022 mature as follows:

	March 31, 2023	
	Less Than 12 Months	More Than 12 Months
Corporate debt securities	$ 10,249	$ —

Total	$ 10,249	$ —

	December 31, 2022	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 12,424	$ —
Corporate debt securities	—	10,186
Total	$ 12,424	$ 10,186

The Company classifies all of its marketable securities as current as they are all either available for sale debt securities or equity investments and are available for current operations.

Convertible senior notes

In September 2019, the Company issued $287.5 million of 1.50% convertible senior notes due September 15, 2026 (the "2026 Convertible Notes,"). The fair value of the 2026 Convertible Notes, which differs from their carrying values, is influenced by interest rates, the Company's stock price and stock price volatility and is determined by prices for the 2026 Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the 2026 Convertible Notes at March 31, 2023 and December 31, 2022 was $326.2 million and $281.7 million, respectively.

Level 3 valuation

The contingent consideration payable is fair valued each reporting period with the change in fair value recorded as a gain or loss within the change in the fair value of deferred and contingent consideration on the consolidated statements of operations. The fair value of the development and regulatory milestones is estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company's estimated development timelines for the acquired product candidate. On March 31, 2023, the weighted average discount rate for the development and regulatory milestones was 7.2% and the weighted average probability of success was 35%. The fair value of the net sales milestones and royalties is determined utilizing an option pricing model with Monte Carlo simulation to simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value. On March 31, 2023, the weighted average discount rate for the net sales milestones and royalties was 12.0% and the weighted average probability of success for the net sales milestones was 50%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the contingent consideration payable for the three months ended March 31, 2023 and March 31, 2022:

	Level 3 liabilities	
	Contingent consideration payable-development and regulatory milestones	Contingent consideration payable-net sales milestones and royalties
Beginning balance as of December 31, 2022	$ 82,500	$ 81,500
Additions	—	—
Change in fair value	3,000	(600)
Payments	—	—
Ending balance as of March 31, 2023	$ 85,500	$ 80,900

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2021	$ 139,300	$ 100,600
Additions	—	—
Change in fair value	(4,600)	(7,100)
Payments	—	—
Ending balance as of March 31, 2022	$ 134,700	$ 93,500

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended March 31, 2023 and December 31, 2022:

	March 31, 2023			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$85,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $331 million 25% - 92% 5.5% - 7.7% 2023 - 2029
Contingent considerable payable- net sales milestones and royalties	$80,900	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 100% 2% - 6% 12% 2025 - 2041

	December 31, 2022			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$82,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $331 million 25% - 92% 6.2% - 8.3% 2023 - 2029
Contingent considerable payable- net sales milestones and royalties	$81,500	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 100% 2% - 6% 11.5% 2025 - 2041

The contingent consideration payables are classified Level 3 liabilities as their valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approaches, including but not limited to, assumptions involving probability adjusted sales estimates for the gene therapy platform and estimated discount rates, the estimated fair value could be significantly higher or lower than the fair value determined.

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at March 31, 2023 and December 31, 2022 consist of the following:

	March 31, 2023	December 31, 2022
Employee compensation, benefits, and related accruals	$ 36,417	$ 62,669
Income tax payable	5,730	4,712
Consulting and contracted research	22,547	38,882
Professional fees	4,216	3,093
Sales allowance	68,136	63,787
Sales rebates	94,296	67,355
Royalties	32,694	40,546

Accounts payable	26,311	27,268
Milestone payable	32,500	—
Other	9,244	12,054
Total	$ 332,091	$ 320,366

6. Capitalization

In August 2019, the Company entered into an At the Market Offering Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald and RBC Capital Markets, LLC (together, the "Sales Agents"), pursuant to which, the Company may offer and sell shares of its common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended. No shares were sold during the three months ended March 31, 2023 and 2022. The remaining shares of the Company's common stock available to be issued and sold, under the At the Market Offering, have an aggregate offering price of up to $93.0 million as of March 31, 2023.

7. Net loss per share

Basic and diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Potentially dilutive securities were excluded from the diluted calculation because their effect would be anti-dilutive.

The following tables set forth the computation of basic and diluted net loss per share:

	Three Months Ended March 31,	
	2023	2022
Numerator		
Net loss	$ (138,959)	$ (126,726)
Denominator		
Denominator for basic and diluted net loss per share	73,729,284	71,215,105
Net loss per share:		
Basic and diluted	$ (1.88)*	$ (1.78)*

* In the three months ended March 31, 2023 and 2022, the Company experienced a net loss and therefore did not report any dilutive share impact.

The following table shows historical dilutive common share equivalents outstanding, which are not included in the above historical calculation, as the effect of their inclusion is anti-dilutive during each period.

	As of March 31,	
	2023	2022
Stock Options	12,100,550	11,751,713
Unvested restricted stock awards and units	3,649,742	2,514,981
Total	15,750,292	14,266,694

8. Stock award plan

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long-Term Incentive Plan, which became effective upon the closing of the Company's initial public offering. On June 8, 2022 (the "Restatement Effective Date"), the Company's stockholders approved the Amended and Restated 2013 Long-Term Incentive Plan (the "Amended 2013 LTIP"). The Amended 2013 LTIP provides for the grant of incentive stock options, nonstatutory stock options, restricted stock units and other stock-based awards. The number of shares of common stock reserved for issuance under the Amended *2013* LTIP is the sum of (A) the number of shares of the Company's common stock (up to 16,724,212 shares) that is equal to the sum of (1) the number of shares issued under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date, (2) the number of shares that remain available for issuance under the 2013 Long-Term

Incentive Plan immediately prior to the Restatement Effective Date and (3) the number of shares subject to awards granted under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date that are outstanding as of the Restatement Effective Date, plus (B) from and after the Restatement Effective Date, an additional 8,475,000 shares of Common Stock. As of March 31, 2023, awards for 6,598,348 shares of common stock are available for issuance under the Amended 2013 LTIP.

There are no additional shares of common stock available for issuance under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan or 2013 Stock Incentive Plan.

In January 2020, the Company's Board of Directors approved the 2020 Inducement Stock Incentive Plan. The 2020 Inducement Stock Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards for, initially, up to at the time, an aggregate of 1,000,000 shares of common stock. Any grants made under the 2020 Inducement Stock Incentive Plan must be made pursuant to the Nasdaq Listing Rule 5635(c)(4) inducement grant exception as a material component of the Company's new hires' employment compensation. In December 2020, the Company's Board of Directors approved an additional 1,000,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. In April 2022, the Company's Board of Directors approved a reduction in the total number of shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan to 1,300,000 shares. In December 2022, the Company's Board of Directors approved an additional 1,700,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. As of March 31, 2023, awards for 1,699,326 shares of common stock were available for issuance under the 2020 Inducement Stock Incentive Plan.

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 four-year period.

Inducement stock option awards

From January 1, 2023 through March 31, 2023, the Company issued a total of 863,410 stock options to various employees. Of those, 98,050 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

Stock option activity—A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value(in thousands)
Outstanding at December 31, 2022	11,502,417	$ 43.33		
Granted	863,410	$ 39.93		
Exercised	(211,561)	$ 28.61		
Forfeited/Cancelled	(53,716)	$ 52.25		
Outstanding at March 31, 2023	12,100,550	$ 43.30	6.38 years	$ 102,959
Vested or Expected to vest at March 31, 2023	3,387,505	$ 47.15	8.44 years	$ 19,149
Exercisable at March 31, 2023	8,348,132	$ 41.68	5.44 years	$ 81,335

The fair value of grants made in the three months ended March 31, 2023 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended

	March 31, 2023
Risk-free interest rate	3.54% - 3.88%
Expected volatility	54%
Expected term	5.5 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the three months ended March 31, 2023 was $21.18 per share.

The expected term of options was estimated based on the Company's historical exercise data and the expected volatility of options was estimated based on the Company's historical stock volatility. The risk-free rate of the options was 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.

Restricted Stock Awards and Restricted Stock Units—Restricted stock awards and restricted stock units are granted subject to certain restrictions, including in some cases service or time conditions (restricted stock). The grant-date fair value of restricted stock awards and restricted stock units, which have been determined based upon the market value of the Company's shares on the grant date, are expensed over the vesting period. From January 1, 2023, through March 31, 2023, the Company issued a total of 1,861,235 restricted stock units to various employees. Of those, 42,755 were inducement grants for restricted stock units, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

The following table summarizes information on the Company's restricted stock awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2022	2,516,336	$ 45.67
Granted	1,861,235	39.55
Vested	(701,066)	46.82
Forfeited	(26,763)	43.62
Unvested at March 31, 2023	3,649,742	$ 42.34

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan (as amended, "ESPP" or the "Plan"), for certain eligible employees. The Plan is administered by the Company's Board of Directors or a committee appointed by the Company's Board of Directors. In June 2021, the Plan was amended to increase the total number of shares available for purchase under the Plan from one million shares to two million shares of the Company's common stock. Employees may participate over a six month period through payroll withholdings and may purchase, at the end of the six month period, the Company's common stock at a purchase price of at least 85% of the closing price of a share of the Company's common stock on the first business day of the offering period or the closing price of a share of the Company's common stock on the last business day of the offering period, whichever is lower. No participant will be granted a right to purchase the Company's common stock under the Plan if such participant would own more than 5% of the total combined voting power of the Company or any subsidiary of the Company after such purchase. For the three months ended March 31, 2023, the Company recorded $0.7 million in compensation expense related to the ESPP.

The Company recorded share-based compensation expense in the statement of operations related to incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock units and the ESPP as follows:

	Three Months Ended March 31,	
	2023	2022
Research and development	$ 15,314	$ 13,034
Selling, general and administrative	13,501	13,555
Total	$ 28,815	$ 26,589

As of March 31, 2023, there was approximately $238.4 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the Company's equity award plans. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.39 years.

9. Debt

Liability for sale of future royalties

In July 2020, the Company entered into the Royalty Purchase Agreement. As RPI's interest is explicitly limited, the $650.0 million cash consideration was classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is being amortized using the effective interest method over the life of the arrangement, in accordance with ASC 470 and ASC 835. The initial annual effective interest rate was determined to be 11.0%. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI and updates the effective interest rate on a quarterly basis. Issuance costs related to the transaction were determined to be immaterial.

The following table shows the activity within the "liability for sale of future royalties- current" and "liability for sale of future royalties- noncurrent" accounts for the three months ended March 31, 2023:

Liability for sale of future royalties- (current and noncurrent)	Three Months Ended March 31, 2023
Beginning balance as of December 31, 2022	$ 757,886
Less: Non-cash royalty revenue payable to RPI	(13,237)
Plus: Non-cash interest expense recognized	18,902
Ending balance	$ 763,551
Effective interest rate as of March 31, 2023	9.8 %

Non-cash interest expense is recorded in the statement of operations within "Interest expense, net".

Senior Secured Term Loan

On October 27, 2022 (the "Closing Date"), the Company entered into a credit agreement (the "Blackstone Credit Agreement") for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that the Company requests such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms, among the Company, certain subsidiaries of the Company (together with the Company, the "Loan Parties") and funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit (collectively, "Blackstone", and such lenders, together with their permitted assignees, the "Lenders" and each a "Lender") and Wilmington Trust, National Association, as the administrative agent for the Lenders.

The Blackstone Credit Agreement provides for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million (the "Initial Loans") and a committed delayed draw term loan facility of up to $150.0 million (the "Delayed Draw Loans" and, together with the Initial Loans, the "Loans") to be funded at the Company's request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplates the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million. The Company capitalized approximately $11.6 million of debt issuance costs which are presented on the balance sheet as a direct deduction from the debt liability and are being amortized over the term of the senior secured term loan facility using the effective interest rate method.

The Loans mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at the Company's option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively. Payment of the Loans are subject to certain premiums specified in the Blackstone Credit Agreement, in each case, from the date of the applicable Loan funded.

All obligations under the Blackstone Credit Agreement are secured, subject to certain exceptions and specified inclusions, by security interests in certain assets of the Loan Parties, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by the Loan Parties in certain of their subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which the Company must remain in compliance. The Blackstone Credit Agreement also requires that the Company maintains consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon the Company consummating acquisitions meeting certain consolidated thresholds described therein. In addition, the Company will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein, including $50.0 million that the Company funded into the reserve account during the quarter ended March 31, 2023. The funds in the reserve account are available to prepay the Loans at any time at the Company's option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for use by the Company subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default.

The following table sets forth total interest expense recognized related to the senior secured term loan:

	Three Months Ended March 31, 2023
Contractual interest expense	$ 9,178
Amortization of debt issuance costs	150
Total	$ 9,328
Effective interest rate	13.2 %

2026 Convertible Notes

In September 2019, the Company issued, at par value, $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by the Company.

The 2026 Convertible Notes are governed by an indenture (the "2026 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2026 Convertible Notes Trustee").

Holders of the 2026 Convertible Notes may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2026 only under the following circumstances:

- during any calendar quarter commencing on or after December 31, 2019 (and only during such calendar quarter), if the last reported sale price of the Company's common stock for at least 20 trading days (whether or not

consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;

- during the five business day period after any five consecutive trading day period (the "measurement period") in which the trading price (as defined in the 2026 Convertible Notes Indenture) per $1,000 principal amount of 2026 Convertible Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company's common stock and the conversion rate on each such trading day;

- during any period after the Company has issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or

- upon the occurrence of specified corporate events.

On or after March 15, 2026, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or any combination thereof at the Company's election.

The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of the Company's common stock per $1,000 principal amount of the 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately $52.52 per share of the Company's common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

The Company is not permitted to redeem the 2026 Convertible Notes prior to September 20, 2023. The Company may redeem for cash all or any portion of the 2026 Convertible Notes, at its option, if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2026 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2026 Convertible Notes, which means that the Company is not required to redeem or retire the 2026 Convertible Notes periodically.

If the Company undergoes a "fundamental change" (as defined in the 2026 Convertible Notes Indenture), subject to certain conditions, holders of the 2026 Convertible Notes may require the Company to repurchase for cash all or part of their 2026 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2026 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2026 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness that is not so subordinated, effectively junior in right of payment to any of the Company's secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by the Company's subsidiaries. The 2026 Convertible Notes Indenture contains customary events of default with respect to the 2026 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the 2026 Convertible Notes when due and payable) occurring and continuing, the 2026 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2026 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2026 Convertible Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, 100% of the principal of and accrued and unpaid interest on the 2026 Convertible Notes will automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately.

The 2026 Convertible Notes consist of the following:

	March 31, 2023	December 31, 2022
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(4,168)	(4,456)
Net carrying amount	$ 283,332	$ 283,044

As of March 31, 2023, the remaining contractual life of the 2026 Convertible Notes is approximately 3.5 years.

The following table sets forth total interest expense recognized related to the 2026 Convertible Notes:

	Three Months Ended March 31,	
	2023	2022
Contractual interest expense	$ 1,069	$ 1,069
Amortization of debt issuance costs	288	283
Total	$ 1,357	$ 1,352
Effective interest rate	1.9 %	1.9 %

In April 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $2.1 million, for the period beginning September 25, 2020 and ending March 14, 2022. This amount is not included in the table above, but was recorded as interest expense, net within the statement of operations for the three months ended March 31, 2022.

2022 Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022, (the “2022 Convertible Notes”). On August 15, 2022, the Company repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 2022 Convertible Notes that was due upon maturity in accordance with the terms of the notes.

The following table sets forth total interest expense recognized related to the 2022 Convertible Notes:

	Three Months Ended March 31,	
	2023	2022
Contractual interest expense	$ —	$ 1,110
Amortization of debt issuance costs	—	181
Total	$ —	$ 1,291
Effective interest rate	— %	3.5 %

10. Commitments and 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 in connection with the Company’s oncology and antibacterial programs. As the Company has discontinued development under its antibacterial program, it no longer expects that milestone and royalty payments from the Company to Wellcome Trust will apply under that agreement, resulting in a change to the total amount of development and regulatory milestone payments the Company may become obligated to pay for this program. Under the oncology program funding agreement, to the extent that the Company develops and commercializes program intellectual property on a for-profit basis itself or in collaboration with a

partner (provided the Company retains overall control of worldwide commercialization), the Company may become obligated to pay to Wellcome Trust development and regulatory milestone payments 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 made the first development milestone payment of $0.8 million to Wellcome Trust under the oncology platform funding agreement during the second quarter of 2016. During the year ended December 31, 2022, the Company incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in accounts payable and accrued expenses on the balance sheet and will be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the termination of dosing of all patients in the study. Additional milestone payments of up to an aggregate of $14.5 million may become payable by the Company to Wellcome Trust under this agreement.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company is obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that is successfully developed and subsequently commercialized or, with respect to collaboration products the Company outlicenses, including Evrysdi, a specified percentage of certain payments the Company receives from its licensee. Since inception, the SMA Foundation has earned $31.6 million, $24.5 million which was paid and $7.1 million which was accrued as of March 31, 2023. The Company's obligation to make such payments would end upon the Company's payment to the SMA Foundation of an aggregate of $52.5 million.

Pursuant to the asset purchase agreement ("Asset Purchase Agreement") between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC) ("Marathon"), Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza up to a specified aggregate maximum amount over the expected commercial life of the asset. In addition, Marathon received a $50.0 million sales-based milestone during the three months ended March 31, 2022.

Pursuant to the Agilis Merger Agreement, Agilis equityholders were previously entitled to receive contingent consideration payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2%-6%. The Company was required to pay $40.0 million of the development milestone payments upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the applicable milestones have been achieved.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders canceled and forfeited their rights under the Agilis Merger Agreement to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in development milestone payments that would have been due upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Agilis Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive $211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Agilis Merger Agreement remain in effect pursuant to their terms, including the Company's obligation to pay up to an aggregate maximum amount of $20.0 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement while excluding the remaining $2.4 million milestone payment that was due and paid upon the passing of the second anniversary of the closing of the Agilis Merger), up to an aggregate maximum amount of $361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich

ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Agilis Merger Agreement.

In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. As a result of such approval, the Company paid the former equityholders of Agilis $50.0 million in accordance with the terms of the Agilis Merger Agreement in the year ended December 31, 2022. As of March 31, 2023, the remaining potential development and regulatory milestones is $331.0 million, and the remaining potential sales milestones is $150.0 million.

On October 25, 2019, the Company completed the acquisition of substantially all of the assets of BioElectron Technology Corporation ("BioElectron"), a Delaware corporation, including certain compounds that the Company has begun to develop as part of its Bio-e platform, pursuant to an asset purchase agreement by and between the Company and BioElectron, dated October 1, 2019 (the "BioElectron Asset Purchase Agreement"). BioElectron was a private company with a pipeline focused on inflammatory and central nervous system (CNS) disorders. The lead program, vatiquinone, is in late stage development for CNS disorders with substantial unmet need and significant commercial opportunity that are complementary to PTC's existing pipeline.

Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of the Company's common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

Subject to the terms and conditions of the Agreement and Plan of Merger, dated as of May 5, 2020 (the "Censa Merger Agreement") by and among the Company, Hydro Merger Sub, Inc., the Company's wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC (such merger pursuant thereto, the "Censa Merger"), former Censa securityholders may become entitled to receive contingent payments from the Company based on (i) the achievement of certain development and regulatory milestones up to an aggregate maximum amount of $217.5 million for sepiapterin's two most advanced programs and receipt of a priority review voucher from the FDA as set forth in the Censa Merger Agreement, (ii) $109.0 million in development and regulatory milestones for each additional indication of sepiapterin, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $160.0 million, (iv) a percentage of annual net sales during specified terms, ranging from single to low double digits of the applicable net sales threshold amount, and (v) any sublicense fees paid to the Company in consideration of any sublicense of Censa's intellectual property to commercialize sepiapterin, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees. Pursuant to the Censa Merger Agreement, the Company has the option to pay the initial $30.0 million development milestone, for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU, if achieved, in cash or shares of the Company's common stock.

In February 2023, the Company completed enrollment of its Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. The Company has decided to exercise its option to pay the $30.0 development milestone in shares of its common stock less any cash payments made to Censa securityholders who are not accredited investors, certain cash payments made to former Censa optionholders and cash in lieu of fractional shares as calculated in accordance with the Censa Merger Agreement. Pursuant to the Censa Merger Agreement, the shares of common stock will be issued at a price of $44.9748 per share, or the volume-weighted average price of the Company's common stock on the Nasdaq Global Select Market for the 30 consecutive trading days immediately prior to the second business day prior to the date that the milestone was achieved. The $30.0 million development milestone is recorded in accounts payable and accrued expense on the Company's consolidated balance sheet as of March 31, 2023.

The Company also has the Tegsedi-Waylivra Agreement for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. Akcea is entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement.

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. Additionally, the Company has royalty payments associated with Translarna, Emflaza, and Upstaza net product revenue, payable quarterly or annually in accordance with the terms of the related agreements.

From time to time in the ordinary course of its business, the Company is subject to claims, legal proceedings and disputes. The Company is not currently aware of any material legal proceedings against it.

11. Revenue recognition

Net product sales

The Company views its operations and manages its business in one operating segment.

During the three months ended March 31, 2023 and 2022, net product sales outside of the United States were $133.0 million and $81.2 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $115.1 million and $79.2 million of the net product sales outside of the United States for the three months ended March 31, 2023 and 2022, respectively. During the three months ended March 31, 2023, and 2022, net product sales in the United States were $54.6 million and $48.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $54.6 million, $44.6 million, and $25.9 million of the net product sales, respectively. During the three months ended March 31, 2022, two countries, the United States and Brazil, accounted for at least 10% of the Company's net product sales, representing $48.6 million and $25.8 million of the net product sales, respectively. For the three months ended March 31, 2023 and 2022, the Company had a total of two and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

As of March 31, 2023 and December 31, 2022, the Company does not have a contract liabilities balance related to net product sales, and has not made significant changes to the judgments made in applying ASC Topic 606.

Collaboration and Royalty revenue

In November 2011, the Company and the SMA Foundation entered into the SMA License Agreement with Roche. Under the terms of the SMA License Agreement, Roche acquired an exclusive worldwide license to the Company's SMA program.

Under the SMA License 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 $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

The SMA program currently has one approved product, Evrysdi, which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. As of March 31, 2023, the Company does not have any remaining research and development event milestones that can be received. The remaining potential sales milestones that can be received is $250.0 million.

For the three months ended March 31, 2023 and 2022, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial.

In addition to research and development and sales milestones, the Company is eligible to receive up to double-digit royalties on worldwide annual net sales of a commercial product under the SMA License Agreement. For the three months ended March 31, 2023 and 2022, the Company has recognized $30.8 and $18.9 million of royalty revenue related to Evrysdi, respectively.

Manufacturing Revenue

For the three months ended March 31, 2023, the Company recognized $2.0 million of manufacturing revenue related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. No manufacturing revenue was recognized in the three months ended March 31, 2022. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three months ended March 31, 2023 and 2022.

As of March 31, 2023 and December 31, 2022, the Company has a contract liabilities balance of $0.2 million and $1.4 million, respectively, relating to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. The Company did not have any contract assets for periods ended March 31, 2023 and December 31 2022. For three months ended March 31, 2023, the Company recognized $1.4 million of revenue related to the amounts included in the contract liability balance at the beginning of the period. For the three months ended March 31, 2022, the Company did not recognize any revenues related to the contract liability balance at the beginning of the period.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of March 31, 2023 and December 31, 2022, the aggregate amount of transaction price allocated to remaining performance obligations related to plasmid DNA and AAV vector production for external customers is $0.2 million and $1.4 million, respectively. The Company expects to recognize revenue over the next one year, as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

12. Intangible assets and goodwill

Definite-lived intangibles

Definite lived intangible assets consisted of the following at March 31, 2023 and December 31, 2022:

Definite lived intangibles assets, gross	Ending Balance at December 31, 2022	Additions	Reclass from Indefinite Lived to Definite Lived	Impairment	Foreign currency translation	Ending Balance at March 31, 2023
Emflaza	$ 420,253	$ 17,515	—	—	—	$ 437,768
Waylivra	9,316	—	—	—	170	9,486
Tegsedi	7,109	2,001	—	—	131	9,241
Upstaza	89,550	—	—	—	—	89,550
Total definite lived intangibles, gross	$ 526,228	$ 19,516	$ —	$ —	$ 301	$ 546,045

Definite lived intangibles assets, accumulated amortization	Ending Balance at December 31, 2022	Amortization	Foreign currency translation	Ending Balance at March 31, 2023
Emflaza	$ (266,023)	$ (36,996)	$ —	$ (303,019)
Waylivra	(2,751)	(261)	(52)	(3,064)
Tegsedi	(1,709)	(292)	(36)	(2,037)
Upstaza	(3,420)	(1,866)	—	(5,286)
Total definite lived intangibles, accumulated amortization	$ (273,903)	$ (39,415)	$ (88)	$ (313,406)
Total definite lived intangibles, net				$ 232,639

Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company records the milestone payment when it becomes payable to Marathon and increase the cost basis for the Emflaza rights intangible asset. For the three months ended March 31, 2023 and 2022, total milestone payments of $17.5 million and $62.1 million were recorded, respectively, which included a $50.0 million sales-based milestone during the three months ended March 31, 2022. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis. As of March 31, 2023, a milestone payable to Marathon of $17.5 million was recorded on the balance sheet within accounts payable and accrued expenses.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement related to sales of Waylivra and Tegsedi. In accordance with the guidance for an asset acquisition, the Company records royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra and Tegsedi intangible assets. For the three months ended March 31, 2023 and 2022, royalty payments of $2.0 million and $0.4 million, respectively, were recorded for Tegsedi. As of March 31, 2023, a royalty payable of $2.0 million for Tegsedi was recorded on the balance sheet within accounts payable and accrued expenses. No royalties for Waylivra have been triggered to date.

For the three months ended March 31, 2023 and 2022, the Company recognized amortization expense of $39.4 million and $23.5 million, respectively, related to the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets. The estimated future amortization of the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of March 31, 2023
2023	$ 118,266
2024	33,462
2025	9,702
2026	9,702
2027 and thereafter	61,507
Total	$ 232,639

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2023 is 5.0 years.

Indefinite-lived intangibles

Indefinite lived intangible assets consisted of the following at March 31, 2023 and December 31, 2022:

Indefinite lived intangibles assets	Ending Balance at December 31, 2022	Additions	Reclass from Indefinite Lived to Definite Lived	Impairment	Foreign currency translation	Ending Balance at March 31, 2023
Upstaza	$ 235,766	$ —	$ —	$ —	$ —	$ 235,766
PTC-FA	112,500	—	—	—	—	112,500
PTC-AS	105,300	—	—	—	—	105,300
Total indefinite lived intangibles	$ 453,566	$ —	$ —	$ —	$ —	$ 453,566
Total intangible assets, net						$ 686,205

In connection with the acquisition of the Company's gene therapy platform from Agilis, the Company acquired rights to Upstaza, for the treatment of AADC deficiency. AADC deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. The gene therapy platform also includes PTC-FA, an asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. Additionally, the gene therapy platform includes two other programs targeting CNS disorders, including PTC-AS for Angelman syndrome, a rare, genetic, neurological

disorder characterized by severe developmental delays. As of March 31, 2023, there have been no changes to the indefinite lived intangible assets balance since the year ended December 31, 2022.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of March 31, 2023, there have been no changes to the balance of goodwill since the date of the Agilis Merger. Accordingly, the goodwill balance as of March 31, 2023 is $82.3 million.

13. Subsequent events

The Company has evaluated subsequent events and transactions through the filing date. There were no material events that impacted the consolidated financial statements or disclosures.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis is meant to provide material information relevant to an assessment of the financial condition and results of operations of our company, including an evaluation of the amounts and certainty of cash flows from operations and from outside resources, so as to allow investors to better view our company from management's perspective. The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2022 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 21, 2023, as amended, or our 2022 Annual Report. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q and Part I, Item 1A. (Risk Factors) of our 2022 Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. Our strategy is to leverage our strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe that this allows us to maximize value for all of our stakeholders. We have a portfolio pipeline that includes several commercial products and product candidates in various stages of development, including clinical, pre-clinical and research and discovery stages, focused on the development of new treatments for multiple therapeutic areas for rare diseases relating to neurology, metabolism and oncology.

Corporate Updates

Global Commercial Footprint

Global DMD Franchise

We have two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged two years and older and in Russia for the treatment of nmDMD in patients aged two years and older. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. During the quarter ended March 31, 2023, we recognized $115.1 million in net sales from Translarna. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. During the quarter ended March 31, 2023, we recognized $54.6 million in net sales from Emflaza.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In June 2022, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2023. In February 2023, we submitted a marketing authorization renewal request to the EMA. This marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18-month, placebo-controlled trial, followed by an 18-month open-label extension, which we refer to together as Study 041. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Within the placebo-controlled trial, Translarna showed a statistically significant treatment benefit across the entire intent to treat population as assessed by the 6-minute walk test, assessing ambulation and

endurance, and in lower-limb muscle function as assessed by the North Star Ambulatory Assessment, a functional scale designed for boys affected by DMD. Additionally, Translarna showed a statistically significant treatment benefit across the intent to treat population within the 10-meter run/walk and 4-stair stair climb, each assessing ambulation and burst activity, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the primary analysis group, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. We expect an opinion from the Committee for Medicinal Products for Human Use in the second quarter of 2023.

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations. In order to commence commercial sale of product pursuant to our Translarna marketing authorization in any particular country in the EEA, we must finalize pricing and reimbursement negotiations with the applicable government body in such country. As a result, our commercial launch will continue to be on a country-by-country basis. We also have made, and expect to continue to make, product available under early access programs, or EAP programs, both in countries in the EEA and other territories. Our ability to negotiate, secure and maintain reimbursement for product under commercial and EAP programs can be subject to challenge in any particular country and can also be affected by political, economic and regulatory developments in such country.

There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, or if our product label is materially restricted, or if Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the United States Food and Drug Administration, or FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. We followed the FDA's recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Study 045 did not meet its pre-specified primary endpoint. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-submission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We then had an informal meeting with the FDA, during which we discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, we plan to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

Upstaza™ (eladocagene exuparvovec)

We have a pipeline of gene therapy product candidates for rare monogenic diseases that affect the CNS, including Upstaza for the treatment of Aromatic L-Amino Decarboxylase, or AADC, deficiency, a rare central nervous system, or CNS, disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene, for patients 18 months and older within the EEA. In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. We are also preparing a biologics license application, or BLA, for Upstaza for the treatment of AADC deficiency in the United States. In October 2022, we held a Type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. At such meeting, the FDA asked for additional bioanalytical data in support

of comparability between the drug product used in the clinical studies and the commercial drug product. We completed these analyses and provided the results to the FDA for review. We received initial feedback from the FDA on these data and we are in the process of responding to the FDA's queries. This could result in a shift in timing of our expected BLA submission from the second quarter to the third quarter of 2023.

Tegsedi® (inotersen) and Waylivra™ (volanesorsen)

We hold the rights for the commercialization of Tegsedi and Waylivra for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to a Collaboration and License Agreement, or the Tegsedi-Waylivra Agreement, dated August 1, 2018, by and between us and Akcea Therapeutics, Inc., or Akcea, a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, European Union, or EU, and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and we continue to make Tegsedi available in certain other countries within Latin America and the Caribbean through EAP programs. In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome, or FCS, in Brazil and we began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy, or FPL. Waylivra has also received marketing authorization in the EU for the treatment of FCS.

Evrysdi® (risdiplam)

We also have a spinal muscular atrophy, or SMA, collaboration 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. The SMA program has one approved product, Evrysdi® (risdiplam), which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA and we expect the EMA to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in 2023.

Diversified Development Pipeline

Splicing Platform

In addition to our SMA program, our splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease, or HD. We announced the results from our Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses blood brain barrier at significant levels and that PTC518 was well tolerated. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. We expect interim data from the initial 12-week portion of the Phase 2 study in the second quarter of 2023.

Bio-e Platform

Our Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in our Bio-e platform are vatiquinone and utreloxastat. We initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures in the third quarter of 2020. We have

completed enrollment in this trial after previously experiencing delays in enrollment due to the COVID-19 pandemic. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023. We also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipate results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

Metabolic Platform

The most advanced molecule in our metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. We initiated a registration-directed Phase 3 trial for sepiapterin for PKU in the third quarter of 2021 with the primary endpoint in the study of achieving statistically-significant reduction in blood Phe level. The primary analysis population includes those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. In January 2023, we announced preliminary data from the Part 1 run-in phase of this trial, including that the mean reduction in blood Phe levels in an initial cohort of subjects during the Part 1 would be recognized as clinically meaningful if maintained in Part 2 of the trial. We expect results from Part 2 of this trial to be available in May 2023.

Oncology Platform

Unesbulin is our most advanced oncology agent. We completed our Phase 1 trials evaluating unesbulin in leiomyosarcoma, or LMS, and diffuse intrinsic pontine glioma, or DIPG, in the fourth quarter of 2021. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. We expect to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG in the fourth quarter of 2023.

Multi-Platform Discovery

In addition, we have a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

COVID 19 Impact

The global pandemic caused by a strain of novel coronavirus, COVID-19, has impacted the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to materially adversely affect our business, financial condition, results of operations, and prospects. For additional information, see “Item 1A. Risk Factors - *We face risks related to health epidemics and other widespread outbreaks of contagious disease, which have previously, and may once again, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID 19) pandemic, which disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, the COVID 19 pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.*” in our 2022 Annual Report.

Funding

The success of our products and any other product candidates we may develop, depends largely on obtaining and maintaining reimbursement from governments and third-party insurers. Our revenues are primarily generated from sales of Translarna for the treatment of nmDMD in countries where we were able to obtain acceptable commercial pricing and reimbursement terms and in select countries where we are permitted to distribute Translarna under our EAP programs and from sales of Emflaza for the treatment of DMD in the United States. We have also recognized revenue from sales of Upstaza for the treatment of AADC deficiency in the EEA and have recognized revenue associated with milestone and

royalty payments from Roche pursuant to a License and Collaboration Agreement, or the SMA License Agreement, by and among us, Roche and, for the limited purposes set forth therein, the SMA Foundation, under our SMA program.

To date, we have financed our operations primarily through our offering of the 1.50% convertible senior notes due 2026, or the 2026 Convertible Notes, our public offerings of common stock in February 2014, in October 2014, in April 2018, in January 2019, and in September 2019, the common stock issued in our "at the marketing offering", our initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement (as defined below), net proceeds from our borrowings under the Blackstone Credit Agreement (as defined below), private placements of our convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We have relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since 2022. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement, under our SMA program.

In October 2022, we entered into a credit agreement, or the Blackstone Credit Agreement, for fundings of up to $950.0 million consisting of a committed loan facility consisting of a senior secured term loan facility funded on October 27, 2022, or the Closing Date, in the aggregate principal amount of $300.0 million, and a delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions, and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' (as defined below) agreement to provide such additional financing and to mutual agreement on terms among us and certain of our subsidiaries, or, collectively with us, the Loan Parties, and funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit, or collectively, Blackstone, and such lenders, together with their permitted assignees, the Lenders, and Wilmington Trust, National Association, as the administrative agent for the Lenders.

The 2026 Convertible Notes consist of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

In August 2019, we entered into an At the Market Offering Sales Agreement, or the Sales Agreement, with Cantor Fitzgerald and RBC Capital Markets, LLC, or together, the Sales Agents, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. During the three months ended March 31, 2023, we did not issue or sell any shares of common stock pursuant to the Sales Agreement. The remaining shares of our common stock available to be issued and sold, under the Sales Agreement, have an aggregate offering price of up to $93.0 million as of March 31, 2023.

As of March 31, 2023, we had an accumulated deficit of $2,795.9 million. We had a net loss of $139.0 million and $126.7 million for the three months ended March 31, 2023 and 2022, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing, including expanding our direct manufacturing capabilities at our leased biologics manufacturing facility and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur ongoing research and development expenses for our products and product candidates, including our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing

authorization in. We are also preparing and anticipate submitting a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States. We are in the process of responding to the FDA's queries and this could result in a shift in timing of our expected BLA submission for Upstaza from the second quarter to the third quarter of 2023. These efforts may significantly impact the timing and extent of our commercialization expenses.

We may seek to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, which would increase our future capital requirements.

We expect to pay the former equityholders of Agilis $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency. We are in the process of responding to the FDA's queries and this could result in a shift in timing of our expected BLA submission for Upstaza from the second quarter to the third quarter of 2023. In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and pursuant to the Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, Censa and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders. We have decided to exercise our option to pay this milestone in shares of our common stock less any cash payments made to Censa securityholders who are not accredited investors, certain cash payments made to former Censa optionholders and cash in lieu of fractional shares as calculated in accordance with the Censa Merger Agreement. Pursuant to the Censa Merger Agreement, the shares of common stock will be issued at a price of $44.9748 per share, or the volume-weighted average price of our common stock on the Nasdaq Global Select Market for the 30 consecutive trading days immediately prior to the second business day prior to the date that the milestone was achieved. We also expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate in cash upon the potential achievement in 2023 of certain development and regulatory milestones relating to sepiapterin.

We also have certain significant contractual obligations and commercial commitments that require funding and we have disclosed these items under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations-Funding Obligations" in our 2022 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2023. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. Accordingly, we may 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 our commercialization efforts.

Financial operations overview

Revenues

Net product revenues. To date, our net product revenues have consisted primarily of sales of Translarna for the treatment of nmDMD in territories outside of the United States and sales of Emflaza for the treatment of DMD in the United States. We recognize revenue when performance obligations with customers have been satisfied. Our performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when our customer obtains control of the product, which is typically upon delivery. We invoice customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of invoice date. We determine the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods not yet provided. As we have identified only one distinct performance obligation, the transaction price is allocated entirely to the product sale. In determining the transaction price, a significant financing component does not exist since the timing from when we

deliver product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

We record product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained. For the three months ended March 31, 2023 and 2022, net product sales outside of the United States were $133.0 million and $81.2 million, respectively consisting of sales of Translarna, Tegsedi, Waylivra and Upstaza. Translarna net revenues made up $115.1 million and $79.2 million of the net product sales outside of the United States for the three months ended March 31, 2023 and 2022, respectively. For the three months ended March 31, 2023 and 2022, net product sales in the United States were $54.6 million and $48.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of our net product sales, representing $54.6 million, $44.6 million, and $25.9 million of net product sales, respectively. During the three months ended March 31, 2022, two countries, the United States and Brazil, accounted for at least 10% of our net product sales, representing $48.6 million and $25.8 million of net product sales, respectively.

In relation to customer contracts, we incur costs to fulfill a contract but do not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. We consider any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Roche and the SMA Foundation Collaboration. In November 2011, we entered into the SMA License Agreement pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our SMA program with the SMA Foundation. The research component of this agreement terminated effective December 31, 2014. We are eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product. As of March 31, 2023, we had recognized a total of $210.0 million in milestone payments and $203.7 million royalties on net sales pursuant to the SMA License Agreement. As of March 31, 2023, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of March 31, 2023 are $250.0 million upon achievement of certain sales events.

For the three months ended March 31, 2023 and 2022, the amounts relating to collaboration revenue related to the SMA License Agreement were immaterial. There were no milestones triggered from Roche in the three months ending March 31, 2023 and 2022.

For the three months ended March 31, 2023 and 2022, we have recognized $30.8 million and $18.9 million of royalty revenue, respectively, related to Evrysdi.

In July 2020, we entered into a Royalty Purchase Agreement with RPI 2019 Intermediate Finance Trust, or RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, or the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI 42.933%, or the Assigned Royalty Payment, of our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement in consideration for $650.0 million. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payment.

Manufacturing Revenue. We have manufacturing services related to the production of plasmid deoxyribonucleic acid, or DNA, and adeno-associated virus, or AAV, vectors for gene therapy applications for external customers. Performance obligations vary but may include manufacturing plasmid DNA and/or AAV vectors, material testing, stability studies, and other services related to material development. The transaction prices for these arrangements are fixed and include amounts stated in the contracts for each promised service. Typically, the performance obligations within a manufacturing contract are highly interdependent, in which case, we will combine them into a single performance obligation. We have determined that the assets created have no alternative use to us, and we have an enforceable right to payment for the performance completed to date, therefore revenue related to these services are recognized over time and is measured using an output method based on performance of manufacturing milestones completed to date.

Manufacturing service contracts may also include performance obligations related to project management services or obtaining materials from third parties. We have determined that these are separate performance obligations for which revenue is recognized at the point in time the obligation is performed. For performance obligations related to obtaining third party materials, we have determined that we are the principal as we have control of the materials and have discretion in setting the price. Therefore, we recognize revenue on a gross basis related to obtaining third party materials.

Certain arrangements require a portion of the contract consideration to be received in advance at the commencement of the contract, and such advance payment is initially recorded as a contract liability. A contract asset may be recognized in the event our satisfaction of performance obligations outpaces customer billings.

For the three months ended March 31, 2023, we recognized $2.0 million of manufacturing revenue related to plasmid DNA and AAV vector production for external customers. No manufacturing revenue was recognized in the three months ended March 31, 2022. As of March 31, 2023 and December 31, 2022, the aggregate amount of transaction price allocated to remaining performance obligations related to plasmid DNA and AAV vector production for external customers is $0.2 million and $1.4 million, respectively.

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 share-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, IT, human resources and other support functions, 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 fluctuate in connection with our ongoing activities, particularly in connection with Study 041 and other studies for Translarna for the treatment of nmDMD, our activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs and performance of our post-marketing requirements imposed by regulatory agencies with respect to our products. 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 products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs, for the three months ended March 31, 2023 and 2022.

	Three Months Ended March 31,	
	2023	2022
	(in thousands)	
Global DMD Franchise	$ 18,406	$ 17,694
Metabolic	58,681	15,756
Gene Therapy	45,527	42,570
Bio-e	12,894	14,864
Oncology	7,070	6,255
Splicing	17,974	14,397
Emvododstat for COVID-19	891	2,348
Discovery	33,681	26,194
Total research and development	$ 195,124	$ 140,078

The successful development of our products and 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 products and product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our products or product candidates that we are developing or may develop in the future, including our ability to negotiate pricing and reimbursement terms acceptable to us;
- 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 our products or product candidates could mean a significant change in the costs and timing associated with the development of that product or 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 any of our products or product candidates or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. In addition, the uncertainty with respect to the duration, nature and extent of negative impacts of the COVID-19 pandemic and responsive measures relating thereto on our ability to successfully enroll our current and future clinical trials, has caused us to experience delays, and may cause us to experience further delays, in our clinical trials and regulatory submissions.

Selling, general and administrative expense

Selling, general and administrative expenses consist primarily of salaries and other related costs for personnel, including share-based compensation expenses, in our executive, legal, business development, commercial, finance, accounting, information technology and human resource functions. Other selling, 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, accounting services and miscellaneous selling costs.

We expect that selling, general and administrative expenses will increase in future periods in connection with our continued efforts to commercialize our products, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest expense, net

Interest expense, net consists of interest expense from the liability for the sale of future royalties related to the Royalty Purchase Agreement, the 2026 Convertible Notes outstanding, the $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022 outstanding, the Blackstone Credit Agreement, offset by interest income earned on investments.

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 generally accepted accounting principles in the United States. 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.

During the three months ended March 31, 2023, there were no material changes to our critical accounting policies as reported in our 2022 Annual Report.

Results of operations

Three months ended March 31, 2023 compared to three months ended March 31, 2022

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2023 and 2022.

(in thousands)	Three Months Ended March 31, 2023	2022	Change 2023 vs. 2022
Net product revenue	$ 187,557	$ 129,832	$ 57,725
Collaboration revenue	6	7	(1)
Royalty revenue	30,831	18,896	11,935
Manufacturing revenue	1,988	—	1,988
Cost of product sales, excluding amortization of acquired intangible asset	14,144	10,135	4,009
Amortization of acquired intangible asset	39,415	23,473	15,942
Research and development expense	195,124	140,078	55,046
Selling, general and administrative expense	86,914	73,271	13,643
Change in the fair value of deferred and contingent consideration	2,400	(11,700)	14,100
Interest expense, net	(27,331)	(23,514)	(3,817)
Other income (expense), net	9,956	(11,855)	21,811
Income tax expense	(3,969)	(4,835)	866

Net product revenues. Net product revenues were $187.6 million for the three months ended March 31, 2023, an increase of $57.7 million, or 44%, from $129.8 million for the three months ended March 31, 2022. The increase in net product revenue was primarily due to an increase in net product sales of Translarna and Emflaza. Translarna net product revenues were $115.1 million for the three months ended March 31, 2023, an increase of $35.9 million, or 45%, compared to $79.2 million for the three months ended March 31, 2022. These results were driven by treatment of new patients and continued geographic expansion. Emflaza net product revenues were $54.6 million for the three months ended March 31, 2023, an

increase of $6.0 million, or 12%, compared to $48.6 million for the three months ended March 31, 2022. These results reflect new patients, high compliance, and broader access.

Collaboration revenues. Collaboration revenues was $6.0 thousand for the three months ended March 31, 2023, a decrease of $1.0 thousand, or 14%, from $7.0 thousand for the three months ended March 31, 2022. The activity for collaboration revenue was immaterial for the three months ended March 31, 2023, and 2022.

Royalty revenue. Royalty revenue was $30.8 million for the three months ended March 31, 2023, an increase of $11.9 million, or 63%, from $18.9 million for the three months ended March 31, 2022. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended March 31, 2023 as compared to the three months ended March 31, 2022. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Manufacturing revenue. Manufacturing revenues were $2.0 million for the three months ended March 31, 2023. The increase is due to the manufacturing services related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. No manufacturing revenue was recognized for the three months ended March 31, 2022.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $14.1 million for the three months ended March 31, 2023, an increase of $4.0 million, or 40%, from $10.1 million for the three months ended March 31, 2022. Cost of product sales consist primarily of royalty payments associated with Emflaza, Translarna, and Upstaza net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, costs associated with Emflaza, Translarna, and Upstaza products sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increase in net product revenue and royalty revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $39.4 million for the three months ended March 31, 2023, an increase of $15.9 million, or 68%, from $23.5 million for the three months ended March 31, 2022. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. The amortization increase is primarily related to additional Marathon contingent payments.

Research and development expense. Research and development expense was $195.1 million for the three months ended March 31, 2023, an increase of $55.0 million, or 39%, from $140.1 million for the three months ended March 31, 2022. The increase in research and development expenses is primarily related to increased investment in research programs and advancement of the clinical pipeline. The increase in R&D expenses includes the achievement of a $30.0 million success-based development milestone for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU.

Selling, general and administrative expense. Selling, general and administrative expense was $86.9 million for the three months ended March 31, 2023, an increase of $13.6 million, or 19%, from $73.3 million for the three months ended March 31, 2022. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was a loss of $2.4 million for the three months ended March 31, 2023, a change of $14.1 million, or over 100%, from a gain of $11.7 million for the three months ended March 31, 2022. The change is related to the fair valuation of the potential future consideration to be paid to former equityholders of Agilis as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Interest expense, net. Interest expense, net was $27.3 million for the three months ended March 31, 2023, an increase of $3.8 million, or 16%, from $23.5 million for the three months ended March 31, 2022. The increase in interest expense, net was primarily due to interest expense recorded from the liability for the sale of future royalties related to the Royalty Purchase Agreement and the Blackstone Credit Agreement.

Other income (expense), net. Other income was $10.0 million for the three months ended March 31, 2023, a change of $21.8 million, or over 100%, from other expense, net of $11.9 million for the three months ended March 31, 2022. The change in other income (expense) resulted primarily from unrealized foreign exchange gains of $8.3 million and unrealized foreign exchange losses of $3.0 million for the three months ended March 31, 2023 and 2022, respectively. The change in other income (expense) also resulted from unrealized gains of $2.2 million and unrealized losses of $6.5 million relating to the equity investments still held at the reporting date for the three months ended March 31, 2023 and 2022, respectively. The change in other income (expense), net also was a result of unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. of $1.0 million and $1.5 million, respectively, for the three months ended March 31, 2022. The change in other income (expense), net related to our equity investments and convertible debt security in ClearPoint Neuro, Inc. for the three months ended March 31, 2023, were immaterial.

Income tax expense. Income tax expense was $4.0 million for the three months ended March 31, 2023, a decrease of $0.9 million, or 18%, compared to income tax expense of $4.8 million for the three months ended March 31, 2022. The decrease in income tax expense is attributable to the deduction provided by IRC Section 250, as well as the projected utilization of foreign tax credits. We also incur income tax expenses in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

As a growing commercial-stage biopharmaceutical company, we are engaging in significant commercialization efforts for our products while also devoting a substantial portion of our efforts on research and development related to our products, product candidates and other programs. To date, our product revenue has been primarily attributable to sales of Translarna for the treatment of nmDMD in territories outside of the United States and from Emflaza for the treatment of DMD in the United States. Our ongoing ability to generate revenue from sales of Translarna for the treatment of nmDMD is dependent upon our ability to maintain our marketing authorizations in Brazil, Russia and in the EEA and secure market access through commercial programs following the conclusion of pricing and reimbursement terms at sustainable levels in the member states of the EEA or through EAP Programs in the EEA and other territories. The marketing authorization requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is subject to the specific obligation to conduct Study 041. Our ability to generate product revenue from Emflaza will largely depend on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors.

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, our “at the market offering” of our common stock, proceeds from the Royalty Purchase Agreement, the private placements of our preferred stock, collaborations, bank and institutional lender debt, 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 expect to continue to incur significant expenses and operating losses for at least the next fiscal year. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2019, we entered into the Sales Agreement, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an “at the market offering” as defined in Rule 415(a)(4) promulgated under the Securities Act. See “Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Corporate Updates—Funding” for additional information.

In September 2019, we closed a private offering of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026 including the full exercise by the initial purchasers of an option to purchase an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net

proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

In July 2020, we entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment in consideration for $650.0 million.

In October 2022, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms.

The Blackstone Credit Agreement provides for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million and a committed delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplates the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million.

The Loans mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively. Payment of the Loans are subject to certain premiums specified in the Blackstone Credit Agreement, in each case, from the date the applicable Loan is funded.

All obligations under the Blackstone Credit Agreement are secured, subject to certain exceptions and specified inclusions, by security interests in certain assets of the Loan Parties, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by the Loan Parties in certain of their subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which we must remain in compliance. The Blackstone Credit Agreement also requires that we maintain consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon our consummating acquisitions meeting certain consolidated thresholds described therein. In addition, we will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein, including $50.0 million that we funded into the reserve account during the quarter ended March 31, 2023. The funds in the reserve account are available to prepay the Loans at any time at our option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for our use subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default.

Cash flows

As of March 31, 2023, we had cash, cash equivalents and marketable securities of $286.3 million.

The following table provides information regarding our cash flows and our capital expenditures for the periods indicated.

	Three Months Ended March 31,	
(in thousands)	**2023**	**2022**
Cash (used in) provided by:		
Operating activities	(29,491)	(97,404)
Investing activities	(28,971)	49,043
Financing activities	4,094	1,168

Net cash used in operating activities was $29.5 million for the three months ended March 31, 2023 and $97.4 million for the three months ended March 31, 2022. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $29.0 million for the three months ended March 31, 2023 and net cash provided by investing activities was $49.0 million for the three months ended March 31, 2022. Cash used in investing activities for the three months ended March 31, 2023, was primarily related to the acquisition of product rights and purchases of fixed assets, partially offset by net sales and redemption of marketable securities. Cash provided by investing activities for the three months ended March 31, 2022 was primarily due to net sales and redemption of marketable securities, offset by acquisition of product rights.

Net cash provided by financing activities was $4.1 million for the three months ended March 31, 2023 and $1.2 million for the three months ended March 31, 2022. Cash provided by financing activities for the three months ended March 31, 2023 and March 31, 2022 were primarily attributable to cash received from the exercise of options, partially offset by payments on our finance lease principal and debt issuance costs related to our senior secured term loan.

Funding requirements

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with the research and development of our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in. We are also preparing and anticipate submitting a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States. We are in the process of responding to the FDA’s queries and this could result in a shift in timing of our expected BLA submission for Upstaza from the second quarter to the third quarter of 2023. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

- seek to satisfy contractual and regulatory obligations that we assumed through our acquisitions and collaborations;
- execute our commercialization strategy for our products, including initial commercialization launches of our products, label extensions or entering new markets;
- are required to complete any additional clinical trials, non-clinical studies or Chemistry, Manufacturing and Controls, or CMC, assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA, Brazil and Russia for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;
- utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates as well as program materials for third parties;
- initiate or continue the research and development of our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- 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 commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, including our offering of the 2026 Convertible Notes, public offerings and private placements of common stock, our “at the market offering” of our common stock, proceeds from the Royalty Purchase Agreement, net proceeds from our borrowings under the Blackstone Credit Agreement and marketable securities, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. 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.

Our future capital requirements will depend on many factors, including:

- our ability to commercialize and market our products and product candidates that may receive marketing authorization;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and products candidates;
- our ability to maintain the marketing authorization for our products, including in the EEA for Translarna for the treatment of nmDMD and whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- our ability to obtain marketing authorization for Upstaza for the treatment of AADC deficiency in the United States;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic or other potential widespread outbreaks of contagious disease;
- our ability to maintain orphan exclusivity in the United States for Emflaza;
- our ability to successfully complete all post-marketing requirements imposed by regulatory agencies with respect to our products;
- the progress and results of activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for any of our products and for any of our other product candidates

that may receive marketing authorization or any additional territories in which we receive authorization to market Translarna;

- the costs, timing and outcome of regulatory review of our splicing, gene therapy, Bio-e, metabolic and oncology programs and Translarna in other territories;
- our ability to utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates and program materials for third parties;
- our ability to satisfy our obligations under the Blackstone Credit Agreement;
- our ability to satisfy our obligations under the indentures governing the 2026 Convertible Notes;
- the timing and scope of growth in our employee base;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates, including those in our splicing, gene therapy, Bio-e, metabolic and oncology programs;
- revenue received from commercial sales of our products or any of our product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- 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, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisitions of Emflaza, Agilis, our Bio-E platform and Censa and our licensing of Tegsedi and Waylivra; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively.

In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and pursuant to the Censa Merger Agreement, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders. We have decided to exercise our option to pay this milestone in shares of our common stock less any cash payments made to Censa securityholders who are not accredited investors, certain cash payments made to former Censa optionholders and cash in lieu of fractional shares as calculated in accordance with the Censa Merger Agreement. Pursuant to the Censa Merger Agreement, the shares of common stock will be issued at a price of $44.9748 per share, or the volume-weighted average price of our common stock on the Nasdaq Global Select Market for the 30 consecutive trading days immediately prior to the second business day prior to the date that the milestone was achieved.

We expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate in cash upon the achievement of certain development and regulatory milestones in 2023 relating to sepiapterin. We also expect to pay the former equityholders of Agilis $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency. We are in the process of responding to the FDA's queries and this could result in a shift in timing of our expected BLA submission for Upstaza from the second quarter to the third quarter of 2023.

We also have certain significant contractual obligations and commercial commitments that require funding and we have disclosed these items under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations-Funding Obligations" in our 2022 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2023. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. We may need to obtain substantial additional funding in connection with our continuing operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs primarily 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 and product candidates and marketing, distribution or licensing arrangements. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. 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, debt or other financings when needed or on attractive terms, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended March 31, 2023, there were no material changes in our market risk or how our market risk is managed, compared to those disclosed under the heading "Quantitative and Qualitative Disclosures about Market Risk" in our 2022 Annual Report.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2023. The term "disclosure controls and procedures", as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives

and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2023, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended March 31, 2023 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time in the ordinary course of our business, we are subject to claims, legal proceedings and disputes, including as a result of patients seeking to participate in our clinical trials or otherwise gain access to our product candidates. We are not currently aware of any material legal proceedings to which we are a party or of which any of our property is subject.

Item 1A. Risk Factors

We have set forth in Item 1A to our Annual Report on Form 10-K for the year ended December 31, 2022, risk factors relating to our business, our industry, our structure and our common stock. Readers of this Quarterly Report on Form 10-Q are referred to such Item 1A for a more complete understanding of risks concerning us.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1*†	Sponsored Research Agreement, as amended dated as of June 1, 2006, by and between the Registrant and Spinal Muscular Atrophy Foundation
10.2*†	Funding Agreement, dated as of May 26, 2010, by and between the Registrant and The Wellcome Trust Limited
10.3	Consulting Agreement between PTC Therapeutics, Inc. and Stuart W. Peltz (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on March 24, 2023)
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Inline XBRL Instance Document*
101.SCH	Inline XBRL Taxonomy Extension Schema Document*
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document*
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Database*
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document*
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document*
104	The cover page from this Quarterly Report on Form 10-Q, formatted in Inline XBRL

* Submitted electronically herewith.

† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

\+ Management contract, compensatory plan or arrangement.

In accordance with SEC Release 33-8238, Exhibits 32.1 and 32.2 are being furnished and not filed.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

PTC THERAPEUTICS, INC.

Date: April 27, 2023

By: /s/ Emily Hill
Emily Hill
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. 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 10 days after the Effective Date, the Foundation will pay to Company US$400,000;

(b) within 30 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$400,000;

(c) within 30 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$400,000; and

(d) within 30 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$400,000.

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.

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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]

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,

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 one million six hundred thousand US dollars ($1,600,000) in partial support for the Additional Research as follows: (a) eight hundred thousand US dollars ($800,000) within 30 days of the Amendment Effective Date; (b) four hundred thousand US dollars ($400,000) within 30 days of the receiving the Mid-Stage Report; and (c) four hundred thousand US dollars ($400,000) within 30 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

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

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.	**SPINAL MUSCULAR ATROPHY FOUNDATION**
/s/ Stuart Peltz	/s/ Florence A. Eng
By: Stuart Peltz	By: Florence A. Eng
Title: President & CEO	Title: President

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

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 $13,120,140.83.

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 10.2

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. 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 3

MILESTONES, MILESTONE DATES AND TRANCHES

Milestone Number	Milestone Description	Milestone Date	Amount of Tranche
0	Signing of Agreement	Within (twenty) 20 Business Days of the Commencement Date	$ 860,000
1	Identify molecules that have in vivo efficacy and that cross the blood-brain barrier (Months 1 – 6)	6 Months after Commencement Date*	$ 2,239,000
2	Identify a single compound as the Development Candidate (DC) (Months 7 – 18)	18 Months after Commencement Date*	$ 2,298,000
3	Data package sufficient for an IND application (Months 19-30)	30 Months after Commencement Date*	N/A

*** in accordance with the provisions of Clause 2.**

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:

(1) First enrolment of a subject in a Phase 1 Clinical Trial of a Product: 0.1x the Trust Contribution.

(2) First enrolment of a subject in a Phase 2 Clinical Trial of a Product: 1x the Trust Contribution.

(3) First enrolment of a subject in a Phase 3 Clinical Trial of a Product (with option to delay to as provided below for an additional 1x payment): 2x the Trust Contribution.

(4) Regulatory Approval of a Product: 2.5x the Trust Contribution.

(5) Provided, that the third and fourth milestones above (for the Phase 3 Trial and Regulatory Approval) shall be payable only in the event the Trust Contribution represents at least 80% of the proposed $5.4 million US funding amount.

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 = $540,000

(2) Phase 2 milestone amount = $5.4 million

(3) Phase 3 milestone amount = $10.8 million

(4) Regulatory Approval milestone amount = $13.5 million

(5) Total of all milestone amounts= $30,240,000

iii) Worked Example: assumes Trust funds $5.4 million and PTC defers at Phase 3:

(1) Phase 1 milestone amount = $540,000

(2) Phase 2 milestone amount = $5.4 million

(3) Phase 3 milestone amount = $16.2 million (deferred as provided below)

(4) Regulatory Approval milestone amount = $13.5 million

(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 31.1

CERTIFICATIONS

I, Matthew B. Klein, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: April 27, 2023

By: /s/ MATTHEW B. KLEIN
Matthew B. Klein
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Emily Hill, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: April 27, 2023

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended March 31, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew B. Klein, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 27, 2023

By: /s/ MATTHEW B. KLEIN
Matthew B. Klein
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended March 31, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Emily Hill, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to her knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 27, 2023

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)