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 June 30, 2017

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 Number)
100 Corporate Court South Plainfield, NJ	**07080**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000
(Registrant's telephone number, including area code)

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes ☑ No ☐

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

Large accelerated filer ☐	Accelerated filer ☑
Non-accelerated filer ☐ (Do not check if a smaller reporting company)	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 August 4, 2017, there were 41,389,046 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 Form10-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 expectations with respect to our acquisition of all rights to EMFLAZA™ (deflazacort) from Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, including with respect to our ability to realize the anticipated benefits of the acquisition (including with respect to future revenue generation and contingent payments to Marathon based on annual net sales);

- our expectations with respect to our commercial launch of EMFLAZA for the treatment of Duchenne muscular dystrophy, or DMD, in the United States, which is still in its initial phases, including with respect to our ability to optimize distribution channels and commercial matters in a timely manner;

- 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 EMFLAZA for the treatment of DMD in the United States and for Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or EEA, and other countries in which we have or may obtain regulatory approval, or there exist significant reimbursed early access programs;

- the anticipated period of market exclusivity for EMFLAZA for the treatment of DMD in the United States under the Orphan Drug Act of 1983, or Orphan Drug Act, and the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act;

- our ability to complete the United States Food and Drug Administration, or FDA, post-marketing requirements to the marketing authorization of EMFLAZA;

- our ability to satisfy our obligations under the terms of the credit and security agreement with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and certain other financial institutions as lenders thereunder;

- our ability to resolve the matters set forth in the Refuse to File letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, including whether filing our NDA over protest with the FDA will result in a timely or successful review of our NDA, and whether we will be required to perform additional clinical and non-clinical trials or analyses at significant cost;

- our ability to enroll, fund, and 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 European Medicines Agency, or EMA, and by the trial's deadline;

- our ability to maintain our marketing authorization of Translarna for the treatment of nmDMD in the EEA (which is subject to the specific obligation to conduct and submit the results of Study 041 to the EMA and is also subject to annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA);

- the timing and scope of our continued commercialization of Translarna as a treatment for nmDMD in the EEA or other territories outside of the United States;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

- our estimates regarding the potential market opportunity for Translarna and EMFLAZA, including the size of eligible patient populations and our ability to identify such patients;

- 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 of Translarna for the treatment of nmDMD, aniridia, and Dravet syndrome/CDKL5, each caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our cancer stem cell program, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- the rate and degree of market acceptance and clinical utility of Translarna and EMFLAZA;
- 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 timing of, and our ability to obtain additional marketing authorizations for, Translarna and our other product candidates;
- the ability of Translarna, EMFLAZA and our other product candidates to meet existing or future regulatory standards;
- our ability to maintain the current label under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD, whether pursuant to our Phase 2 study of Translarna for nmDMD in pediatric patients, or otherwise;
- the timing of our planned closures of extension trials for Translarna for the treatment of nmCF;
- the potential receipt of revenues from future sales of Translarna, EMFLAZA and other product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD in the countries in which we have or may obtain regulatory approval and EMFLAZA for the treatment of DMD in the United States;
- the potential impact that enrollment, funding and completion of Study 041 may have on our revenue growth;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver Translarna and EMFLAZA 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 Translarna, EMFLAZA and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our other regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to pursue development of Translarna for additional indications;
- our ability to advance our earlier stage programs, including our cancer stem cell program;
- our plans to pursue research and development of other product candidates;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets;
- the potential advantages of Translarna and EMFLAZA;
- our intellectual property position;
- the impact of government laws and regulations;
- our competitive position; and
- our expectations with respect to the development and regulatory status of our product candidates and program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or the SMA Foundation, and our estimates regarding future revenues from achievement of milestones in that program.

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 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, 2016 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 per share data)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 133,011	$ 58,321
Marketable securities	48,058	173,345
Trade receivables, net	33,767	24,929
Inventory	6,912	—
Prepaid expenses and other current assets	5,224	4,691
Total current assets	226,972	261,286
Fixed assets, net	6,839	7,429
Intangible assets, net	148,138	—
Deposits and other assets	1,129	630
Total assets	$ 383,078	$ 269,345
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 63,996	$ 48,759
Deferred revenue	2,602	—
Other current liabilities	1,387	865
Total current liabilities	67,985	49,624
Deferred revenue - long-term	3,828	1,587
Long-term debt	141,242	98,216
Other long-term liabilities	292	335
Total liabilities	213,347	149,762
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 41,304,008 shares at June 30, 2017. Authorized 125,000,000 shares; issued and outstanding 34,169,410 shares at December 31, 2016	41	34
Additional paid-in capital	949,331	856,142
Accumulated other comprehensive income (loss)	1,999	(1,485)
Accumulated deficit	(781,640)	(735,108)
Total stockholders' equity	169,731	119,583
Total liabilities and stockholders' equity	$ 383,078	$ 269,345

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues:				
Net product revenue	$ 47,891	$ 15,437	$ 74,334	$ 34,314
Collaboration and grant revenue	71	196	176	214
Total revenues	47,962	15,633	74,510	34,528
Operating expenses:				
Cost of product sales	758	—	797	—
Research and development	30,835	28,827	58,198	60,226
Selling, general and administrative	28,866	23,366	54,365	49,304
Total operating expenses	60,459	52,193	113,360	109,530
Loss from operations	(12,497)	(36,560)	(38,850)	(75,002)
Interest expense, net	(3,008)	(2,060)	(5,227)	(4,016)
Other expense, net	(1,820)	(387)	(2,139)	(1,107)
Loss before income tax expense	(17,325)	(39,007)	(46,216)	(80,125)
Income tax (expense) benefit	(150)	93	(316)	(22)
Net loss attributable to common stockholders	$ (17,475)	$ (38,914)	$ (46,532)	$ (80,147)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	39,621,738	34,000,333	36,978,528	33,959,751
Net loss per share—basic and diluted (in dollars per share)	$ (0.44)	$ (1.14)	$ (1.26)	$ (2.36)

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net loss	$ (17,475)	$ (38,914)	$ (46,532)	$ (80,147)
Other comprehensive loss:				
Unrealized (loss) gain on marketable securities, net of tax	(9)	(40)	(31)	618
Foreign currency translation gain (loss)	2,884	(159)	3,515	1,467
Comprehensive loss	$ (14,600)	$ (39,113)	$ (43,048)	$ (78,062)

See accompanying unaudited notes.

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

	Six Months Ended June 30,	
	2017	**2016**
Cash flows from operating activities		
Net loss	$ (46,532)	$ (80,147)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,448	1,664
Change in valuation of warrant liability	3	47
Non-cash interest expense	3,274	2,941
Loss on disposal of asset	47	—
Amortization of premiums on investments	365	1,140
Amortization of debt issuance costs	185	147
Share-based compensation expense	16,914	17,651
Benefit for deferred income taxes	—	(244)
Unrealized foreign currency transaction losses, net	1,648	963
Changes in operating assets and liabilities:		
Inventory, net	(2,806)	—
Prepaid expenses and other current assets	(416)	1,163
Trade receivables, net	(6,762)	(8,480)
Deposits and other assets	(463)	(170)
Accounts payable and accrued expenses	12,452	(3,435)
Other liabilities	457	1
Deferred revenue	4,604	587
Net cash used in operating activities	(15,582)	(66,172)
Cash flows from investing activities		
Purchases of fixed assets	(579)	(275)
Purchases of marketable securities	(19,467)	(46,256)
Sale and redemption of marketable securities	144,357	89,645
Acquisition, including transaction costs	(76,424)	—
Net cash provided by investing activities	47,887	43,114
Cash flows from financing activities		
Proceeds from exercise of options	535	34
Proceeds from shares issued under employee stock purchase plan	557	—
Debt issuance costs related to secured term loan	(432)	—
Proceeds from issuance of secured term loan	40,000	—
Net cash provided by financing activities	40,660	34
Effect of exchange rate changes on cash	1,725	660
Net increase in cash and cash equivalents	74,690	(22,364)
Cash and cash equivalents, beginning of period	58,321	58,022
Cash and cash equivalents, end of period	$ 133,011	$ 35,658
Supplemental disclosure of cash information		
Cash paid for interest	$ 2,474	$ 2,263
Cash paid for income taxes	$ 334	$ 264
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized (loss) gain on marketable securities, net of tax	$ (31)	$ 618

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
June 30, 2017
In thousands (except per share data unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") was incorporated as a Delaware corporation on March 31, 1998. PTC is a global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using its expertise in RNA biology. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology.

The Company has two products, Translarna™ (ataluren) and EMFLAZA™ (deflazacort). Translarna received marketing authorization from the European Commission in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients age five years and older in the 31 member states of the European Economic Area, or EEA. EMFLAZA is approved in the United States for the treatment of Duchenne muscular dystrophy, or DMD, in patients five years and older. nmDMD (including DMD) is a rare, life threatening disorder.

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, or EMA, of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. This marketing authorization is further subject to the specific obligation to conduct and submit the results of a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna in the approved patient population. The final report on the trial and open-label extension is to be submitted by the Company to the EMA by the end of the third quarter of 2021. The Company refers to the trial and open-label extension together as Study 041.

The marketing authorization in the EEA was last renewed in June 2017 and is effective, unless extended, through August 5, 2018. The renewal was based on the Company's commitment to conduct Study 041 and the totality of the clinical data available from its trials and studies of Translarna for the treatment of nmDMD, including the safety and efficacy results of the Phase 2b and Phase 3 clinical trials. The primary efficacy endpoint was not achieved in either trial within the pre-specified level of statistical significance.

In June 2014, the Company initiated reimbursed early access programs, or EAP programs, for Translarna for nmDMD patients in selected territories in the EEA and recorded its first sales of Translarna in the third quarter of 2014 pursuant to an EAP program. In December 2014, the Company recorded its first commercial sales in Germany. As of June 30, 2017, Translarna was available in over 25 countries on a commercial basis or pursuant to an EAP program. The Company expects to expand its launch activities across the EEA pursuant to the marketing authorization granted by the EMA throughout 2017 and future years, subject to continued renewal of its marketing authorization following annual EMA reassessments and successful completion of pricing and reimbursement negotiations. Concurrently, the Company plans to continue to pursue EAP programs in select countries where those mechanisms exist, both within the EEA and in other countries that will reference the marketing authorization in the EEA.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, the Company filed a New Drug Application, or NDA, over protest with the United States Food and Drug Administration, (the "FDA"). The FDA has granted a standard review for the NDA and has set a target review date under the Prescription Drug User Fee Act, or PDUFA, of October 24, 2017, and has tentatively scheduled an advisory committee meeting on September 28, 2017 to facilitate its review. The PDUFA date is the goal date for the FDA to complete its review of the NDA, however, such date is not binding on the agency and there can be no assurance that the FDA will complete its review of the Company's NDA by the PDUFA goal date. Filing over protest is a procedural path permitted by FDA regulations that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission. The NDA, which seeks approval of Translarna for the treatment of nmDMD in the United States, was initially submitted by the Company in December 2015. In February 2016, following the initial submission, the Company received a Refuse to File letter from the FDA regarding the NDA. The FDA stated in the Refuse to File letter that the NDA was not sufficiently complete to permit a substantive review. Specifically, the Company was notified in the letter that, in the view of the FDA, both the Phase 2b and Phase 3 ACT DMD trials were negative and do not provide substantial evidence of effectiveness and that the NDA did not contain adequate information regarding the abuse potential of Translarna. Additionally, the FDA stated that the Company had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. During July 2016, the Company appealed the Refuse to File decision via the formal dispute resolution process within FDA's Center for Drug Evaluation and Research; however, this appeal was denied by the FDA's Office of Drug Evaluation I in October 2016.

On March 2, 2017, the Company announced that the primary and secondary endpoints were not achieved in ACT CF, the Company's Phase 3 double-blind, placebo-controlled, 48-week clinical trial comparing Translarna to placebo in nonsense mutation cystic fibrosis, or nmCF, patients six years of age or older not receiving chronic inhaled aminoglycosides. The safety profile of Translarna in the ACT CF study was consistent with previous studies and no new safety signals were identified. Based on the results of ACT CF, the Company has discontinued its current clinical development of Translarna for nmCF and has begun to close ongoing extension studies of Translarna for the treatment of nmCF. The Company has withdrawn its type II variation submission with the EMA, which sought approval of Translarna for the treatment of nmCF in the EEA.

On April 20, 2017, the Company completed its acquisition of all rights to EMFLAZA, or the Transaction. EMFLAZA is approved in the United States for the treatment of DMD in patients five years and older. The Transaction was completed pursuant to an asset purchase agreement, dated March 15, 2017, as amended on April 20, 2017, (the "Asset Purchase Agreement"), by and between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon. The transaction was accounted for as an asset acquisition. The assets acquired by the Company in the Transaction include intellectual property rights related to EMFLAZA, inventories of EMFLAZA, and certain contractual rights related to EMFLAZA. The Company assumed certain liabilities and obligations in the Transaction arising out of, or relating to, the assets acquired in the Transaction.

Upon the closing of the Transaction, the Company paid to Marathon total upfront consideration comprised of $75.0 million in cash, funded through cash on hand, and 6,683,598 shares of the Company's common stock. The number of shares of common stock issued at closing was determined by dividing $65.0 million by the volume weighted average price per share of the Company's common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing. Marathon will be 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, and a single $50.0 million sales-based milestone, in each case subject to the terms and conditions of the Asset Purchase Agreement.

As of June 30, 2017, the Company had an accumulated deficit of approximately $781.6 million. The Company has financed its operations to date primarily through the private offering in August 2015 of 3.00% convertible senior notes due 2022 (see Note 9), public offerings of common stock in February 2014 and October 2014, its initial public offering of common stock in June 2013, private placements of its convertible preferred stock, collaborations, bank debt and convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates. Since 2014, 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, and in May 2017, the Company began to recognize revenue generated from net sales of EMFLAZA for the treatment of DMD in the United States.

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, 2016 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 16, 2017 (the "2016 Form 10-K"). Additional significant accounting policies adopted during the six month period ended June 30, 2017 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of June 30, 2017 and for the three and six months ended June 30, 2017 and 2016 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, 2016 and notes thereto included in the 2016 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2017 and for the three and six months ended June 30, 2017 and 2016 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three and six month periods ended June 30, 2017 are not necessarily indicative of the results to be expected for the year ended December 31, 2017 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, certain accruals related to the Company's research and development expenses, stock-based compensation, valuation procedures for the convertible notes, allowance for doubtful accounts, inventory, acquired intangible assets, 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.

Inventory and cost of product sales

In January 2017, the European Commission granted an annual renewal of the Company's marketing authorization for Translarna for the treatment of nmDMD. Until this renewal, the Company had considered the authorization to be subject to risk and did not capitalize productions costs in inventory as it was not probable that such costs would be recovered. With the renewal, the Company now considers recovery of the costs to be probable and began capitalizing production costs in inventory, effective January 1, 2017. Production costs will be expensed as cost of product sales when the related products are sold. The costs for a portion of the inventory available for sale was expensed as research and development costs prior to the January 2017 annual renewal of the Translarna marketing authorization and as such the cost of products sold and related gross margins are not necessarily indicative of future cost of products sold and gross margin.

In April 2017, the Company completed the Transaction (refer to Note 11). EMFLAZA, both in tablet and suspension form, received approval from the FDA on February 9, 2017 as a treatment for DMD in patients five years of age and older. The Company began the commercialization of EMFLAZA in the United States shortly after the acquisition was completed. The Company utilizes third parties for the commercial distribution of EMFLAZA, including a third-party logistics company to warehouse EMFLAZA as well as specialty pharmacies to sell and distribute EMFLAZA to patients. All of the Company's manufacturing needs for EMFLAZA are fulfilled pursuant to exclusive supply agreements assumed by the Company upon close of the acquisition of EMFLAZA. Production costs will be expensed as cost of product sales when the related products are sold.

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. Translarna and EMFLAZA product 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.

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

	June 30, 2017	December 31, 2016
Work in progress	$ 941	$ —
Finished goods	5,971	—
Total inventory	$ 6,912	$ —

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. The Company has not recorded any inventory write downs as of the current period. 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.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, storage costs, amortization of the acquired intangible asset and royalty payments associated with net product sales.

Revenue recognition

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

Net product sales

Prior to the second quarter of 2017, the Company's net product sales consisted of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. The Company has now established a pattern of collectability and, since January 2015, the Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition—Products.

The Company has recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program. Orders for Translarna are generally received from hospital and retail pharmacies and the Company's third-party partner distributors. Revenue is recognized when risk of ownership has transferred. The Company's third-party partner distributors act as intermediaries between the Company and end users and do not typically stock significant quantities of Translarna. The ultimate payor for Translarna is typically a government authority or institution or a third-party health insurer.

In May 2017, the Company began the commercialization of EMFLAZA in the U.S. The Company recorded product revenue related to the sales of EMFLAZA in the U.S. in accordance with ASC 605-15, when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured. Due to the early stage of the product launch, the Company determined that it was not able to reliably make certain estimates, including returns, necessary to recognize product revenue upon shipment to distributors. As a result, the Company recorded net product revenue for EMFLAZA using a deferred revenue recognition model (sell-through). Under the deferred revenue model, the Company does not recognize revenue until EMFLAZA is shipped to an end-user. The Company will continue to evaluate when, if ever, it has sufficient volume of historical activity and visibility into the distribution channel, in order to reasonably make all estimates required under ASC 605 to recognize revenue upon shipment to its distributors.

The Company records revenue net of estimated third-party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

Collaboration and grant 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.

The Company evaluates all contingent consideration earned, such as a milestone payment, using the criteria as provided by ASC 605-28, Revenue Recognition—Milestone Method. At the inception of a collaboration arrangement, the Company evaluates if a milestone payment is substantive. The criteria requires that (1) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered a substantive milestone and will be recognized as revenue in the period that the milestone is achieved. The Company recognizes royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

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 allowance for doubtful accounts was $0.7 million as of June 30, 2017 and $0.7 million as of December 31, 2016.

Business combinations and asset acquisitions

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen (as adopted in the current period under Accounting Standards Update (ASU) No. 2017-01, "Business Combinations (Topic 805): Clarifying the Definition of a Business"; see "Impact of recently adopted accounting standards" and Note 11 for further details) to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen is met, the transaction is accounted for as an asset acquisition. If the screen is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs, which would meet the requirements of a business. If determined to be a business combination, the Company accounts for the transaction under the acquisition method of accounting as indicated in ASC Topic 805, "Business Combinations", which requires the acquiring entity in a business combination to recognize the fair value of all assets acquired, liabilities assumed, and any non-controlling interest in the acquiree and establishes the acquisition date as the fair value measurement point. Accordingly, the Company recognizes assets acquired and liabilities assumed in business combinations, including contingent assets and liabilities, and non-controlling interest in the acquiree based on the fair value estimates as of the date of acquisition. In accordance with ASC 805, the Company recognizes and measures goodwill as of the acquisition date, as the excess of the fair value of the consideration paid over the fair value of the identified net assets acquired.

The consideration for the Company's business acquisitions includes future payments that are contingent upon the occurrence of a particular event or events. The obligations for such contingent consideration payments are recorded at fair value on the acquisition date. The contingent consideration obligations are then evaluated each reporting period. Changes in the fair value of contingent consideration obligations, other than changes due to payments, are recognized as a (gain) loss on fair value remeasurement of contingent consideration in the condensed consolidated statements of operations.

If determined to be an asset acquisition, the Company accounts for the transaction under ASC 805-50, which requires the acquiring entity in an asset acquisition to recognize assets (net assets) based on the cost to the acquiring entity on a relative fair value basis, which includes transaction costs in addition to consideration given. No gain or loss is recognized as of the date of acquisition unless the fair value of noncash assets given as consideration differs from the assets' carrying amounts on the acquiring entity's books. Consideration transferred that is noncash will be measured based on either the cost (which shall be measured based on the fair value of the consideration given) or the fair value of the assets (net assets) acquired, whichever is more reliably measurable. Goodwill is not recognized in an asset acquisition and any excess consideration transferred over the fair value of the net assets acquired is allocated to the identifiable assets based on relative fair values.

Contingent consideration payments in asset acquisitions are recognized when the contingency is resolved and the consideration is paid or becomes payable (unless the contingent consideration meets the definition of a derivative, in which case the amount becomes part of the basis in the asset acquired). Upon recognition of the contingent consideration payment, the amount is included in the measuring of the cost of the acquired asset or group of assets.

Finite-lived intangible assets

The Company records the fair value of purchased intangible assets with finite useful lives as of the transaction date of a business combination or asset acquisition. Purchased intangible assets with finite useful lives are amortized to their estimated residual values over their estimated useful lives. The Company evaluates the finite-lived intangible assets for impairment whenever events or changes in circumstances indicate the reduction in the fair value below their respective carrying amounts. If the Company determines that an impairment has occurred, a write-down of the carrying value and an impairment charge to operating expenses in the period the determination is made is recorded. In addition, the remaining estimated useful life of the finite-lived intangible asset would be reassessed.

Recently issued accounting standards

In May 2014, the FASB issued ASU No. 2014-9, "Revenue from Contracts with Customers (Topic 606)". ASU No. 2014-9 will eliminate transaction- and industry-specific revenue recognition guidance under current GAAP and replace it with a principle-based approach for determining revenue recognition. ASU No. 2014-9 includes the required steps to achieve the core principle that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects

the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU will also require additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. With the issuance of ASU No. 2015-14 in August 2015, the FASB deferred the effective date of the revenue recognition guidance to reporting periods beginning after December 15, 2017. Early adoption of the standard is permitted but not before the original effective date, which was for reporting periods beginning after December 15, 2016. With the issuance of ASU No. 2016-8 in March 2016 and ASU No. 2016-10 in April 2016, the FASB further amended guidance on recording revenue on a gross versus a net basis and on identifying performance obligations and licensing, respectively. The Company expects to use the modified retrospective approach to adopt this guidance when effective. The Company continues to evaluate the effect that the updated standard, as well as additional amendments, may have on its consolidated financial statements and accompanying notes. The Company's implementation approach includes performing a detailed review of key contracts representative of the product being sold and services provided and assessing the conformance of historical accounting policies and practices with the standard. Because the standard may impact the Company's business processes, systems and controls, the Company has initiated the development of a comprehensive change management project plan to guide the implementation.

In January 2016, the FASB issued ASU No. 2016-1, "Financial Instruments — Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities". This standard enhances the reporting model for financial instruments, which includes amendments to address aspects of recognition, measurement, presentation and disclosure. The new guidance affects all reporting organizations (whether public or private) that hold financial assets or owe financial liabilities. ASU 2016-1 is effective for years beginning after December 15, 2017, including interim periods within those fiscal years. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-1 will have on its consolidated financial statements and accompanying notes.

In February 2016, the FASB issued ASU No. 2016-2, "Leases (Topic 842)". This standard will require organizations that lease assets with lease terms of more than 12 months to recognize assets and liabilities for the rights and obligations created by those leases on their balance sheets. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. The standard is effective for public companies for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018, with early adoption permitted. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-2 will have on its consolidated financial statements and accompanying notes.

In June 2016, the FASB issued ASU No. 2016-13, "Financial Instruments — Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments". This standard requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected. This standard is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. The Company expects to adopt this guidance when effective and is assessing what effect the adoption of ASU 2016-13 will have on its consolidated financial statements and accompanying notes.

In August 2016, the FASB issued ASU No. 2016-15, "Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments". This standard clarifies the presentation of certain specific cash flow issues in the Statement of cash flows. The standard is effective for public companies who are SEC filers for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2017, with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on the Company's consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, "Statement of Cash Flows (Topic 230): Restricted Cash". This standard requires entities to show the changes in the total of cash, cash equivalents, restricted cash and restricted cash equivalents in the statement of cash flows and no longer present transfers between cash and cash equivalents and restricted cash and restricted cash equivalents in the statement of cash flows. This standard is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2017, including interim periods within those years, with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on the Company's consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, "Stock Compensation (Topic 718): Scope of Modification Accounting". This standard clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as a modification, with entities applying the modification accounting guidance if the value, vesting conditions or classification of the award changes. In addition to all disclosures about modifications that are required under the current guidance, entities will be also required to disclose that compensation expense has not changed if applicable. This standard is effective for public companies who are SEC filers for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2017, with early adoption permitted, including any interim period for which financial statements have not yet been issued or made available for issuance.

The guidance will be applied prospectively to awards modified on or after the adoption date. The Company expects to adopt this guidance when effective.

Impact of recently adopted accounting pronouncements

In November 2015, the FASB issued ASU No. 2015-17, “Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes”. This standard requires all deferred tax assets and liabilities to be classified as non-current on the balance sheet instead of separating deferred taxes into current and non-current amounts. In addition, valuation allowance allocations between current and non-current deferred tax assets are no longer required because those allowances also will be classified as non-current. This standard is effective for public companies for annual periods beginning after December 15, 2016. The Company adopted the guidance on January 1, 2017 on a prospective basis. As the Company’s deferred tax assets is provided with full valuation allowance as of June 30, 2017, adoption of this standard did not have a significant impact on the Company's financial statements.

In March 2016, the FASB issued ASU No. 2016-9, “Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting”. This standard requires the recognition of all income tax effects of awards in the income statement when the awards vest or are settled, with Additional Paid in Capital (APIC) pools to be eliminated. In addition, the standard will increase the amount an employer can withhold to cover income taxes on awards and still qualify for the exception to liability classification for shares used to satisfy the employer’s statutory income tax withholding obligation as well as allowing companies to elect whether to account for forfeitures of share-based payments by recognizing forfeitures of awards as they occur or estimating the number of awards expected to be forfeited and adjusting the estimate when it is likely to change, as is currently required. This standard is effective for public companies for fiscal years beginning after December 15, 2016 and interim periods within those years. The Company adopted the guidance on January 1, 2017 and on a prospective basis, the Company will record all excess tax benefits and deficiencies as income tax expense or benefit. Due to the Company's history of operating losses, the adoption did not result in changes to the Company's Net loss or Retained earnings. In connection with the adoption of ASU 2016-9, the Company made a policy election to continue its methodology for estimating its forfeiture rate.

In January 2017, the FASB issued ASU No. 2017-01, "Business Combinations (Topic 805): Clarifying the Definition of a Business". This standard changed the definition of a business to help entities determine whether a set of transferred assets and activities is a business. This standard is effective for public companies for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years, with early adoption permitted. The Company has elected to early adopt ASU No. 2017-01 and apply the guidance to the Transaction, which is being accounted for as an asset acquisition under the revised guidance.

3. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provides guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules 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 investments are reflected in the accompanying financial statements at fair value. The carrying amount of receivables, accounts payable and accrued expenses, and debt approximates fair value due to the short-term nature of those instruments.

Fair value of certain marketable 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 investments, the Company used the fair value as determined by its investment advisors using observable inputs other than quoted prices.

The Company reviews its investments on a periodic basis for other-than-temporary impairments. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may have a significant adverse effect on the fair value of the investment.

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 June 30, 2017 and December 31, 2016:

	June 30, 2017			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 48,058	$ —	$ 48,058	$ —
Warrant liability	$ 4	$ —	$ —	$ 4
Stock appreciation rights liability	$ 1,102	$ —	$ —	$ 1,102

	December 31, 2016			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 173,345	$ —	$ 173,345	$ —
Warrant Liability	$ 1	$ —	$ —	$ 1
Stock appreciation rights liability	$ 865	$ —	$ —	$ 865

No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the periods ended June 30, 2017 and December 31, 2016.

The following is a summary of marketable securities accounted for as available-for-sale securities at June 30, 2017 and December 31, 2016:

	June 30, 2017			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
Commercial paper	$ —			$ —
Corporate debt securities	48,093	—	(35)	48,058
Government obligations	—	—	—	—
	$ 48,093	$ —	$ (35)	$ 48,058

	December 31, 2016			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
Commercial paper	$ 12,919	$ 47	$ —	$ 12,966
Corporate debt securities	153,240	52	(103)	153,189
Government obligations	7,188	2	—	7,190
	$ 173,347	$ 101	$ (103)	$ 173,345

At June 30, 2017 and December 31, 2016, the Company held securities with an unrealized loss position that were not considered to be other-than-temporarily impaired as the Company has the ability to hold such investments until recovery of their fair value. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity. As of June 30, 2017, the Company had $0.03 million in realized gains resulting from the sale of investments. As of December 31, 2016, the Company did not have any realized gains/losses from the sale of marketable securities.

Marketable securities on the balance sheet at June 30, 2017 and December 31, 2016 mature as follows:

	June 30, 2017	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ —	$ —
Corporate debt securities	48,058	—
Government obligations	—	—
Total Marketable securities	$ 48,058	$ —

	December 31, 2016	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 12,966	$ —
Corporate debt securities	137,196	15,993
Government obligations	7,190	—
Total Marketable securities	$ 157,352	$ 15,993

The Company classifies all of its securities as current as they are all available for sale and are available for current operations.

Level 3 valuation

The warrant liability is classified in Other long-term liabilities on the Company's consolidated balance sheets. The warrant liability is marked-to-market each reporting period with the change in fair value recorded as a gain or loss within Other expense, net, on the Company's consolidated statements of operations until the warrants are exercised, expire or other facts and circumstances lead the warrant liability to be reclassified as an equity instrument. The fair value of the warrant liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

The stock appreciation rights (SARs) liability is classified in Other liabilities on the Company's consolidated balance sheets. The SARs liability is marked-to-market each reporting period with the change in fair value recorded as compensation expense on the Company's consolidated statements of operations until the SARS vest. The fair value of the SARs liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the warrant liability and SARs liability for the period ended June 30, 2017:

	Level 3 liabilities	
	Warrants	SARs
Beginning balance as of December 31, 2016	$ 1	$ 865
Change in fair value	3	1,301
Payments	—	(1,064)
Ending balance as of June 30, 2017	$ 4	$ 1,102

Fair value of the warrant liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of June 30, 2017 include (i) volatility (69%—70%), (ii) risk free interest rate (1.38%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($18.33), and (v) expected life (2.10—2.23 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2016 include (i) volatility (62%-67%), (ii) risk free interest rate (0.62%—1.34%), (iii) strike price ($128.00—$2,520.00), (iv) fair value of common stock ($10.91), and (v) expected life (0.4—2.7 years).

Fair value of the SARs liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's SARs as of June 30, 2017 include (i) volatility (67%—70%), (ii) risk free interest rate (1.14%—1.47%), (iii) strike price ($6.76-$30.86), (iv) fair value of common stock ($18.33), and (v) expected life (0.5—2.5 years). The significant assumptions used in preparing the option pricing model for valuing the Company's SARs as of December 31, 2016 include (i) volatility (48%-71%), (ii) risk free interest

rate (0.44%—1.47%), (iii) strike price ($6.76—$30.86), (iv) fair value of common stock ($10.91), and (v) expected life (0.0—3.0 years).

4. Other comprehensive income (loss) and accumulated other comprehensive items

Other comprehensive income (loss) includes changes in equity that are excluded from net income (loss), such as unrealized gains and losses on marketable securities.

The following tables summarize other comprehensive income (loss) and the changes in accumulated other comprehensive items for the three and six months ended June 30, 2017:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at March 31, 2017	$ (225)	$ (651)	$ (876)
Other comprehensive (loss) income before reclassifications	(9)	2,884	2,875
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive (loss) income	(9)	2,884	2,875
Balance at June 30, 2017	$ (234)	$ 2,233	$ 1,999

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2016	$ (203)	$ (1,282)	$ (1,485)
Other comprehensive (loss) income before reclassifications	(31)	3,515	3,484
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive (loss) income	(31)	3,515	3,484
Balance at June 30, 2017	$ (234)	$ 2,233	$ 1,999

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at June 30, 2017 and December 31, 2016 consist of the following:

	June 30, 2017	December 31, 2016
Employee compensation, benefits, and related accruals	$ 11,064	$ 13,649
Consulting and contracted research	11,006	11,505
Professional fees	2,252	1,237
Sales allowance and other costs	23,836	13,245
Accounts payable	6,062	6,298
Other	9,776	2,825
	$ 63,996	$ 48,759

6. Warrants

All of the Company's outstanding warrants were classified as liabilities as of June 30, 2017 and December 31, 2016 because they contained non-standard antidilution provisions.

The following is a summary of the Company's outstanding warrants as of June 30, 2017 and December 31, 2016:

	June 30, 2017		
	Warrant shares	Exercise price	Expiration
Common stock	7,030	$ 128.00	2019
Common stock	130	$ 2,520.00	2019

	December 31, 2016		
	Warrant shares	Exercise price	Expiration
Common stock	6,250	$ 128.00	2017
Common stock	7,030	$ 128.00	2019
Common stock	130	$ 2,520.00	2019

7. Net loss per share

Basic earnings per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net loss by the weighted-average number of common shares plus the effect of dilutive potential common shares outstanding during the period.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Numerator				
Net loss	$ (17,475)	$ (38,914)	$ (46,532)	$ (80,147)
Denominator				
Denominator for basic and diluted net loss per share	39,621,738	34,000,333	36,978,528	33,959,751
Net loss per share:				
Basic and diluted	$ (0.44) *	$ (1.14) *	$ (1.26) *	$ (2.36) *

*In the three and six months ended June 30, 2017 and 2016, 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 June 30,	
	2017	2016
Stock Options	7,124,052	5,969,382
Unvested restricted stock awards and units	423,986	274,490
Total	7,548,038	6,243,872

8. Stock award plan

On March 5, 2013, the Company's Board of Directors approved the 2013 Stock Incentive Plan, which provides for the granting of stock option awards, stock appreciation rights, restricted stock, restricted stock units and other stock-based awards in the aggregate of 739,937 shares of common stock. On March 5, 2013, the Board approved a grant of 735,324 shares of restricted stock and 4,613 stock options. There are no additional shares available for issuance under this plan.

In 2009, the Company's shareholders approved the 2009 Equity and Long-Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards, subject to certain adjustments and annual increases. In May 2013, the Company's Board of Directors and stockholders increased by 2,500,000 the number of

shares authorized under the 2009 Equity and Long Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards. There are no additional shares available for issuance under this 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 IPO. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. The number of shares of common stock reserved for issuance under the 2013 Long Term Incentive Plan is the sum of (1) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of each fiscal year until the expiration of the 2013 Long Term Incentive Plan, equal to the lowest of 2,500,000 shares of common stock, 4% of the number of shares of common stock outstanding on the first day of the fiscal year and an amount determined by the Company's Board of Directors. As of June 30, 2017, awards for 425,683 shares of common stock are available for issuance.

From January 1, 2017 through June 30, 2017, the Company issued a total of 1,738,873 stock options to various employees. Of those, 480,550 were inducement grants for non-statutory stock options. The inducement grant awards were made pursuant to the NASDAQ inducement grant exception as a material component of the Company's new hires' employment compensation and not under the 2013 Long Term Incentive Plan.

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, 2016	5,854,316	$ 34.71		
Granted	1,738,873	$ 11.80		
Exercised	(49,770)	$ 10.74		
Forfeited/Cancelled	(419,367)	$ 33.83		
Outstanding at June 30, 2017	7,124,052	$ 29.33	7.51 years	$ 19,807
Vested or Expected to vest at June 30, 2017	3,159,956	$ 25.63	8.66 years	$ 10,427
Exercisable at June 30, 2017	3,738,673	$ 32.94	6.44 years	$ 8,449

The fair value of grants made in the six months ended June 30, 2017 was contemporaneously estimated on the date of grant using the following assumptions:

	Six months ended June 30, 2017
Risk-free interest rate	1.84% — 2.45%
Expected volatility	76%—81%
Expected term	5.04– 10.00 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the six-month period ended June 30, 2017 was $8.07 per share.

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

Restricted Stock Awards—Restricted stock awards 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, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

Restricted Stock Units—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 units, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

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
January 1, 2017	271,651	$ 19.76
Granted	358,194	$ 11.34
Vested	(180,861)	$ 14.19
Forfeited	(24,998)	$ 13.47
Unvested at June 30, 2017	423,986	$ 15.39

Stock Appreciation Rights—Stock appreciation rights (SARs) entitle the holder to receive, upon exercise, an amount of the Company's common stock or cash (or a combination thereof) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of the Company's common stock over the measurement price based on the exercise date.

In May 2016, a total of 897,290 SARs were granted to non-executive employees (the 2016 SARs). The 2016 SARs will vest annually in equal installments over four years and will be settled in cash on each vest date, requiring the Company to remeasure the SARs at each reporting period until vesting occurs. For the period ending June 30, 2017, a total of 213,197 SARs vested and the Company recorded $1.3 million in compensation expense related to the 2016 SARs.

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan ("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 Board. The total number of shares available for purchase under the Plan is one 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 period ending June 30, 2017, the Company issued 107,499 shares of common stock and recorded $0.3 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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Research and development	$ 3,895	$ 4,087	$ 8,362	$ 8,415
Selling, general and administrative	3,990	4,649	8,552	9,236
Total	$ 7,885	$ 8,736	$ 16,914	$ 17,651

As of June 30, 2017, there was approximately $56.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, the 2013 Long Term Incentive Plan and equity awards made pursuant to the NASDAQ inducement grant exception for new hires. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.19 years.

9. Debt

2017 Credit Facility

In May 2017, the Company entered into a credit and security agreement (the "Credit Facility") with MidCap Financial Trust, a Delaware statutory trust (“MidCap”), as administrative agent and MidCap and certain other financial institutions as lenders thereunder (the “Credit Agreement”) that provides for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by the Company on May 5, 2017. The remaining $20.0 million under the senior secured term loan facility will become available to the Company upon its demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120.0 million for the trailing 12 month period. The Company capitalized approximately $0.4 million of debt issuance costs, which were netted against the carrying value of the Credit Facility and will be amortized over the term of the Credit Facility.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%. The Company is obligated to make interest only payments (payable monthly in arrears) through April 30, 2019. Commencing on May 1, 2019 and continuing for the remaining twenty-four months of the facility, the Company will be required to make monthly interest payments and monthly principal payments. The principal payments are to be made based on straight-line amortization of the principal over the twenty-four month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

The Credit Facility is subject to certain financial covenants. As of June 30, 2017, the Company was in compliance with all required covenants.

Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.0% convertible senior notes due 2022 (the "Convertible Notes"). The Convertible Notes bear cash interest at a rate of 3.0% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The Convertible Notes will mature on August 15, 2022, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $145.4 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by the Company.

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

Holders may convert their Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding February 15, 2022 only under the following circumstances:

- during any calendar quarter commencing on or after September 30, 2015 (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 Convertible Notes Indenture) per $1,000 principal amount of 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 February 15, 2022, until the close of business on the business day immediately preceding the maturity date, holders may convert their Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay cash up to the aggregate principal amount of the Convertible Notes to be converted and deliver shares of its common stock in respect of the remainder, if any, of its conversion obligation in excess of the aggregate principal amount of Convertible Notes being converted.

The conversion rate for the Convertible Notes was initially, and remains, 17.7487 shares of the Company's common stock per $1,000 principal amount of the Convertible Notes, which is equivalent to an initial conversion price of approximately $56.34 per share of the Company's common stock.

The Company may not redeem the Convertible Notes prior to August 20, 2018. The Company may redeem for cash all or any portion of the Convertible Notes, at its option, on or after August 20, 2018 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 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the Convertible Notes, which means that the Company is not required to redeem or retire the Convertible Notes periodically.

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

The Convertible Notes Indenture contains customary events of default with respect to the Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the Convertible Notes when due and payable) occurring and continuing, the Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the Convertible Notes Trustee at the request of such holders (subject to the provisions of the Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 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 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 Company accounts for the Convertible Notes as a liability and equity component where the carrying value of the liability component will be valued based on a similar instrument. In accounting for the issuance of the Convertible Notes, the Company separated the Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated convertible feature. The carrying amount of the equity component representing the conversion option was determined by deducting the fair value of the liability component from the par value of the Convertible Notes as a whole. The excess of the principal amount of the liability component over its carrying amount, referred to as the debt discount, is amortized to interest expense over the seven-year term of the Convertible Notes. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The equity component recorded at issuance related to the Convertible Notes is $57.5 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the Convertible Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the Convertible Notes, and transaction costs attributable to the equity component are netted with the equity components in stockholders' equity. Additionally, the Company initially recorded a net deferred tax liability of $22.3 million in connection with the Notes.

The Convertible Notes consist of the following:

Liability component	**June 30, 2017**	**December 31, 2016**
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(2,294)	(2,457)
Less: Debt discount, net(1)	(46,054)	(49,327)
Net carrying amount	$ 101,652	$ 98,216

(1) Included in the consolidated balance sheets within convertible senior notes (due 2022) and amortized to interest expense over the remaining life of the Convertible Notes using the effective interest rate method.

The fair value of the Convertible Notes was approximately $115.8 million as of June 30, 2017. The Company estimates the fair value of its Convertible Notes utilizing market quotations for debt that have quoted prices in active markets. As of June 30, 2017, the remaining contractual life of the Convertible Notes is approximately 5.1 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Contractual interest expense	$ 1,131	$ 1,125	$ 2,241	$ 2,241
Amortization of debt issuance costs	83	75	163	147
Amortization of debt discount	1,674	1,495	3,274	2,941
Total	$ 2,888	$ 2,695	$ 5,678	$ 5,329
Effective interest rate of the liability component	11%	11%	11%	11%

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 cancer stem cell 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 cancer stem cell 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’s first such milestone payment of $0.8 million payable to Wellcome Trust occurred in the second quarter of 2016. Additional milestone payments of up to an aggregate of $22.4 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 may become 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, if the Company outlicenses rights to a collaboration product, a specified percentage of certain payments the Company receives from its licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. The Company’s obligation to make such payments would end upon our payment to the SMA Foundation of a specified amount.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Additionally, the Company has royalty payments associated with Translarna and EMFLAZA product net sales, payable quarterly or annually in accordance with the terms of the related agreements.

11. Emflaza asset acquisition

On April 20, 2017, the Company completed its previously announced acquisition of all rights to EMFLAZA pursuant to an Asset Purchase Agreement, dated March 15, 2017, and amended on April 20, 2017, by and between the Company and Marathon. The assets acquired by the Company in the Transaction include intellectual property rights related to EMFLAZA, inventories of EMFLAZA, and certain contractual rights related to EMFLAZA. The Company assumed certain liabilities and obligations in the Transaction arising out of, or relating to, the assets acquired in the Transaction.

The Company concluded that the EMFLAZA Agreement included inputs and processes that did not constitute a business under the revised guidance of ASU No. 2017-01, which allows for a screen to evaluate if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen is met, the transaction is accounted for as an asset acquisition. The Company determined that substantially all of the fair value is concentrated in the EMFLAZA rights intangible asset and accounted for the transaction as an asset acquisition under ASC 805-50.

The purchase price consisted of total upfront consideration comprised of $75.0 million in cash and 6,683,598 shares of the Company's common stock with a fair value of $75.2 million. In addition, the Company incurred approximately $2.2 million of acquisition costs, which are capitalized in an asset acquisition and included in the total consideration transferred.

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 addition, Marathon has the opportunity to receive a single $50.0 million sales-based milestone. In accordance with the guidance for an asset acquisition, the Company will record the milestone payment when it becomes payable to Marathon and increase the cost basis for the EMFLAZA rights intangible asset.

The following tables present the total purchase consideration and the preliminary allocation of the purchase consideration for the Transaction as of April 20, 2017 (the “Acquisition Date”):

Cash consideration	$	75,000
Fair value of PTC common stock issued to Marathon (6,683,598 shares)		75,190
Acquisition costs		2,163
Total preliminary consideration transferred	$	152,353

Purchase price	$	152,353

Total fair value of tangible assets acquired and liabilities assumed:		
Inventory		3,980
EMFLAZA rights	$	148,373

The EMFLAZA rights intangible asset is being amortized to cost of product sales using the economic use method over its expected useful life of approximately seven years. The method of amortization represents the pattern in which the economic benefits of the asset are expected to be consumed based on future projected cash flows of the intangible asset. Although the Company believes such available information and assumptions are reasonable, given the inherent risks and uncertainties underlying its expectations regarding such future revenues, there is the potential for the Company’s actual results to vary significantly from such expectations. As such, if the pattern as to which the asset will be consumed changes significantly, the related amortization of the intangible assets will change in proportion to the change in revenues. As of June 30, 2017, the Company recognized accumulated amortization of $0.2 million with respect to the EMFLAZA rights intangible asset. The estimated future amortization of the EMFLAZA rights intangible asset is expected to be as follows:

		As of June 30, 2017
2017 (1)	$	1,707
2018		11,695
2019		18,896
2020		25,918
2021 and thereafter		89,922
Total	$	148,138

(1) For the six months ended December 31, 2017.

12. Subsequent events

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

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

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, 2016 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 16, 2017. 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, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a global biopharmaceutical company focused on the discovery, development and commercialization of novel medicines using our expertise in RNA biology. We have discovered all of our compounds currently under development using our proprietary technologies. We plan to continue to develop these compounds both on our own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. Our internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology.

During the quarter ended June 30, 2017, we recognized $45.8 million in sales of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, and $2.1 million in sales of EMFLAZA™ (deflazacort) for the treatment of Duchenne muscular dystrophy, or DMD. Translarna is currently available in over 25 countries on a commercial basis or through a reimbursed early access program, or EAP program. Translarna is an investigational new drug in the United States, or U.S. We hold worldwide commercialization rights to Translarna for all indications in all territories. EMFLAZA is indicated for the treatment of DMD in patients five years of age and older in the U.S.

Corporate Updates

Acquisition of EMFLAZA™ for the treatment of Duchenne muscular dystrophy in the United States

On April 20, 2017, we completed the acquisition of all rights to EMFLAZA pursuant to an asset purchase agreement, dated March 15, 2017 and amended on April 20, 2017, or the Asset Purchase Agreement, by and between us and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon.

Since the founding of the Company nearly twenty years ago, PTC has been committed to fundamentally changing the lives of patients living with DMD. In addition to our historical and continued investment in research and development, this commitment has included raising disease awareness, promoting diagnosis and early intervention, and supporting improved standards of care all with the goal of changing the course of the disease. It is our continuing commitment to the Duchenne community that underpinned the acquisition of EMFLAZA. PTC is committed to make this important therapy available to all eligible patients in the United States and to study its long-term benefits.

Upon the closing of our acquisition of EMFLAZA, we paid to Marathon total upfront consideration comprised of $75.0 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock, which was determined by dividing $65.0 million by the volume weighted average price per share of our common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing. Marathon will be entitled to receive contingent payments from us 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, and a single $50.0 million sales-based milestone, in each case subject to the terms and conditions of the Asset Purchase Agreement.

EMFLAZA, both in tablet and suspension form, received approval from the U.S. Food and Drug Administration, or FDA, on February 9, 2017 as a treatment for DMD in patients five years of age and older. We estimate that there are approximately 9,000 DMD patients in the U.S. aged five years or older. We are obligated to complete certain post-marketing requirements in connection with the FDA's approval, including pre-clinical and clinical safety studies.

We expect that EMFLAZA will have a seven-year exclusive marketing period in the U.S. for the approved indication, commencing on the date of FDA approval, under the provisions of the Orphan Drug Act of 1983, or the Orphan Drug Act, as well as a concurrent five-year exclusive marketing period in the U.S. for the active ingredient in EMFLAZA under the provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. As we presently have no patent rights to protect the approved use of EMFLAZA, we expect to rely on both the five-year Hatch-Waxman Act and seven-year Orphan Drug Act exclusivity periods to commercialize EMFLAZA for the approved indication in the U.S. As the holder of orphan exclusivity, we are required to ensure the availability of sufficient quantities of EMFLAZA to meet the needs of patients. Failure to do so could result in loss of orphan exclusivity in the U.S.

We began the commercialization of EMFLAZA in the U.S. shortly after the acquisition was completed. We utilize third parties for the commercial distribution of EMFLAZA, including a third-party logistics company to warehouse EMFLAZA as well as specialty pharmacies to sell and distribute EMFLAZA to patients. A specialty pharmacy provides us with third-party call center services to provide patient support and financial services, prescription intake and distribution, reimbursement adjudication, and

ongoing compliance support. All of our manufacturing needs for EMFLAZA are fulfilled pursuant to exclusive supply agreements assumed by us upon close of our acquisition of EMFLAZA.

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third-party payors. As part of our commercial launch of EMFLAZA in the U.S., we are engaged in pricing, coverage and reimbursement discussions with third-party payors, such as state and federal governments, including Medicare and Medicaid, managed care providers, private commercial insurance plans and pharmacy benefit management plans. Decisions regarding the extent of coverage and the amount of reimbursement to be provided for EMFLAZA are made on a plan-by-plan, and in some cases, on a patient-by-patient basis.

To date, over 1,200 patients are receiving EMFLAZA from our commercial and bridge programs. We anticipate that coverage and reimbursement decisions by third-party payors, including the processing and adjudication of prescriptions, may vary from weeks to several months. Certain third-party payors may impose additional requirements before approving reimbursement of a prescription, including prior authorization and the requirement to try another therapy first, which would delay our ability to obtain payment for prescriptions for EMFLAZA.

We have been engaging with key stakeholders in the DMD community to understand their needs and to address recent negative publicity and increasing legislative and public scrutiny around pharmaceutical drug pricing in the U.S., in particular with respect to orphan drugs and specifically with respect to EMFLAZA. U.S. government authorities and third-party payors are increasingly attempting to limit or regulate drug prices and reimbursement (including through mandatory discounts under certain government sponsored programs), which may limit our ability to generate revenue from commercial sales of EMFLAZA.

Regulatory, clinical and marketing authorization matters for Translarna in nonsense mutation Duchenne muscular dystrophy

United States. Translarna is an investigational new drug in the U.S. In October 2016, the Office of Drug Evaluation I of the FDA denied our first appeal of the Refuse to File letter issued by the FDA's Division of Neurological Products on February 22, 2016 regarding our New Drug Application, or NDA, for Translarna for the treatment of nmDMD. In the first quarter of 2017, we filed our Translarna NDA via the FDA's file over protest regulations. Filing over protest is a procedural path permitted by FDA regulations that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission.

The FDA has notified us that it has granted a standard review for the Translarna NDA and has set a target review date under the Prescription Drug User Fee Act, or PDUFA, of October 24, 2017, and has tentatively scheduled an advisory committee meeting on September 28, 2017 to facilitate its review. The target review date is not binding on the agency and there can be no assurance that the FDA will complete its review of the Translarna NDA by the target PDUFA date.

There is significant risk that, notwithstanding any dialogue we have had or any further dialogue we may be able to initiate with the FDA, pursuant to the file over protest process or otherwise, the agency will continue to disagree with our interpretation of the results of our Phase 3 clinical trial in nmDMD, or ACT DMD, and the totality of clinical data from our trials, and will not grant marketing authorization for Translarna for the treatment of nmDMD.

For additional information regarding risks to our ability to obtain marketing authorization for Translarna for the treatment of nmDMD in the U.S., see "Item 1A. Risk Factors," including the risk factor titled, "*ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations*."

European Economic Area. In June 2017, the European Commission renewed our marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older in the 31 member states of the European Economic Area, or EEA, and it is effective, unless extended, through August 5, 2018. We received initial marketing authorization from the European Commission in August 2014. The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of continued authorization, which we refer to as the annual EMA reassessment, as well as our satisfaction of any specific obligation or other requirement placed upon the marketing authorization, including Study 041. Study 041 is a three-year clinical trial to confirm the efficacy and safety of Translarna in the approved patient population. The trial is comprised of two stages: an 18-month randomized, double-blind, placebo controlled clinical trial followed by an 18-month open label extension period. We expect to submit the results of Study 041 to the European Medicines Agency, or EMA, by the end of the third quarter of 2021. We expect that as part of the annual EMA assessment, the EMA will consider the ongoing status of Study 041.

As part of our pediatric development commitments under our marketing authorization in the EEA and to support the potential expansion of Translarna's labeling to younger patients with nmDMD, we initiated a Phase 2 pediatric clinical study to evaluate the safety and pharmacokinetics of Translarna in patients two to five years of age. The study, initiated in June 2016, includes a

four-week screening period, a four-week study period, and a 48-week extension period for patients who complete the four-week study period (52 weeks total treatment). By the end of 2017, we intend to submit with the EMA a label-extension request to our marketing authorization in the EEA to include patients from two to up to five years of age, which will include data from this study. However, there can be no assurances that we will successfully obtain such label extension.

For additional information regarding the risks related to the renewal of our marketing authorization in the EEA, see "Item 1A. Risk Factors," including the risk factor titled "*Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon our ability to satisfy the specific obligation to conduct and report the results of Study 041 by September 2021, and, as such, there is ongoing risk that we may be unable to maintain such authorization. If we are unable to obtain renewal of such marketing authorization in any future renewal cycle, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, financial performance and results of operations.*"

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations and system. 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.

For additional information regarding risks to our business arising as a result of matters relating to pharmaceutical pricing and reimbursement of Translarna see "Item 1A. Risk Factors," including the risk factor titled "*Commercialization of Translarna has been in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues, if any. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna in the European Economic Area and other jurisdictions would prevent us from marketing our products in such regions*."

Translarna™ for nonsense mutation cystic fibrosis

On March 2, 2017, we announced that the primary and secondary endpoints were not achieved in ACT CF, our Phase 3 double-blind, placebo-controlled, 48-week clinical trial comparing Translarna to placebo in nmCF patients six years of age or older not receiving chronic inhaled aminoglycosides. The safety profile of Translarna in the ACT CF trial was consistent with previous studies and no new safety signals were identified. Based on the results of ACT CF, we have discontinued our current clinical development of Translarna for nmCF and have begun to close ongoing extension studies of Translarna for the treatment of nmCF. We have withdrawn our type II variation submission with the EMA, which sought approval of Translarna for the treatment of nmCF in the EEA.

Translarna™ for additional indications

Based on its understood mechanism of action, we believe that Translarna may have benefit in the treatment of patients with genetic disorders that arise as a result of a nonsense mutation. We are pursuing studies for Translarna in additional indications: nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5. We recently decided that in the third quarter of 2017 we will stop our study for Translarna in mucopolysaccharidosis type I caused by nonsense mutation, or nmMPS I, as we have encountered difficulties identifying qualified patients for this study, and we determined it was best to move our resources to other areas.

Spinal muscular atrophy program

Our spinal muscular atrophy, or SMA, collaboration is 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. Sunfish, a two-part clinical study in pediatric and adult type 2 and type 3 SMA patients initiated in the fourth quarter of 2016, followed by the initiation of Firefish in the fourth quarter of 2016, a two-part clinical study in infants with type 1 SMA. Both Sunfish and Firefish are investigating the safety, tolerability and efficacy of the compound RG7916 in the applicable patient populations. Part one of each study is a dose-finding study with the primary objectives of evaluating the safety, pharmacokinetics, or PK, and pharmacodynamics of RG7916 in patients and to select the dose for part two of the applicable study. Preliminary interim results from Part 1 of the Sunfish study was presented at the 2017 CureSMA Researcher Meeting at the end of the second quarter of 2017, with the results showing that type 2/3 SMA patients receiving RG7916 demonstrated a dose-dependent increase in SMN2 full length/Δ7 mRNA ratio of ~ 400% versus baseline, as measured in whole blood. We believe that these results provide proof of mechanism for the oral, small molecule SMN2 splicing modifier RG7916. No drug-related adverse events leading to withdrawal have been observed to date for RG7916. Part one of each study is expected to be followed by a pivotal part two

with the primary objective of evaluating the efficacy and safety of RG7916. Commencement of the pivotal part two portion of either Sunfish or Firefish will trigger a single $20 million milestone payment to us from Roche. We anticipate that both Sunfish and Firefish will move into the pivotal second part of the respective study during the second half of 2017. Jewelfish, an open-label study investigating the safety, tolerability, PK, and PK/pharmacodynamic relationship of RG7916 in type 2 and type 3 SMA patients who have been previously treated with a survival of motor neuron 2 (SMN2)-targeting therapy, initiated in the first quarter of 2017.

Cancer stem cell program

A Phase 1 first-in-human, dose-escalation safety and pharmacokinetic open-label clinical study in advanced cancer patients with solid tumors initiated in April 2015 and completed in the first quarter of 2017 for PTC596, our product candidate in the cancer stem cell program. PTC596 was generally well tolerated as a monotherapy, producing systemic concentrations in patients similar to or exceeding those associated with preclinical activity. Though a protocol-defined maximum tolerated dose was not reached, the dose of 10 mg/kg was deemed intolerable due to pill burden and certain excipients that may have contributed to Grade 2 nausea, vomiting, and diarrhea in two of three patients. Data from this study and continued clinical development of PTC596, including reformulation efforts, are expected during 2017.

Funding

The success of Translarna, EMFLAZA, or other product candidates we may develop and/or commercialize, depends largely on obtaining and maintaining reimbursement from governments and third-party insurers.

Since 2015, our revenues have been primarily generated from sales of Translarna for the treatment of nmDMD in territories where we are permitted to distribute Translarna under EAP programs and in countries in the EEA where we were able to obtain acceptable pricing and reimbursement terms.

On April 20, 2017, we completed our acquisition of all rights to EMFLAZA for total upfront consideration comprised of $75.0 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock, which was determined by dividing $65.0 million by the volume weighted average price per share of our common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing.

On May 5, 2017, we entered into a credit and security agreement, or the Credit Agreement, with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto, that provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing 12 month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

To date, we have financed our operations primarily through our offering of 3.00% convertible senior notes due August 15, 2022, or the Convertible Notes offering, our public offerings of common stock in February 2014 and in October 2014, our initial public offering of common stock in June 2013, private placements of our preferred stock, collaborations, bank debt and convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, we began to recognize revenue generated from net sales of EMFLAZA for the treatment of DMD in the United States.

As of June 30, 2017, we had an accumulated deficit of $781.6 million. We had a net loss of $46.5 million and $80.1 million for the six months ended June 30, 2017 and 2016, respectively.

We anticipate that our expenses will increase in connection with our commercialization efforts in the United States, the EEA 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 Study 041 and our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to EMFLAZA in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our cancer stem cell program.

In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing authorization for Translarna for other indications. 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.

With respect to our outstanding Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.5 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company. These costs include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Additionally, we could be forced to expend significant resources in the defense of the pending securities class action lawsuits brought against us and certain of our executives, as described under Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q. See also, "*The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock and lawsuits against us and our officers and directors*" under Part II, Item 1A. Risk Factors - Risks Related to Our Common Stock.

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

To date, our net product sales have consisted primarily of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. We also began the commercialization of EMFLAZA in the U.S. shortly after the completion of the acquisition of all rights to EMFLAZA. Our process for recognizing revenue is described below under "Critical accounting policies and significant judgments and estimates—Revenue recognition".

Roche and the SMA Foundation Collaboration. In November 2011, we entered into a license and collaboration agreement, or licensing agreement, with Roche and the SMA Foundation pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our spinal muscular atrophy program with the SMA Foundation. The research component of this agreement terminated effective December 31, 2014. The licensing agreement included a $30 million upfront payment made in 2011 which was recognized on a deferred basis over the research term, and the potential for up to $460 million in milestone payments and royalties on net sales.

In August 2013, we announced the selection of a development candidate. The achievement of this milestone triggered a $10.0 million payment to us from Roche, which we recorded as collaboration revenue for the year ended December 31, 2013.

In January 2014, we initiated a Phase 1 clinical program, which triggered a $7.5 million milestone payment to us from Roche which we recorded as collaboration revenue for the year ended December 31, 2014.

In November 2014, we announced that our joint development program in Spinal Muscular Atrophy (SMA) with Roche and the SMA Foundation (SMAF) has started a Phase 2 study in adult and pediatric patients. The achievement of this milestone triggered a $10.0 million payment to us from Roche which we recorded as collaboration revenue for the year ended December 31, 2014.

Grant revenue. From time to time, we receive grant funding from various institutions and governmental bodies. The grants are typically for early discovery research, and generally such grant programs last from two to five years.

Research and development expense

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

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;

- employee-related expenses, which include salaries and benefits, including 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 increase in connection with our ongoing activities, particularly in connection with Study 041 for Translarna for the treatment of nmDMD, our studies of Translarna in nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5, activities under our cancer stem cell program, and performance of our FDA post-marketing requirements with respect to EMFLAZA in the United States. 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 tables provide research and development expense for our most advanced principal product development programs, for the three and six months ended June 30, 2017 and 2016.

	Three Months Ended June 30,	
	2017	2016
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 21,008	$ 21,383
Cancer stem cell	682	1,691
Next generation nonsense readthrough	1,334	1,680
EMFLAZA	2,518	—
Other research and preclinical	5,293	4,073
Total research and development	$ 30,835	$ 28,827

	Six Months Ended June 30, 2017	
	2017	2016
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 40,429	$ 43,975
Cancer stem cell	2,126	3,731
Next generation nonsense readthrough	2,762	3,577
EMFLAZA	2,598	—
Other research and preclinical	10,283	8,943
Total research and development	$ 58,198	$ 60,226

The successful development of Translarna and our other 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 and to obtain or maintain marketing authorizations we have or may receive from our products and product candidates;

· 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 Translarna or any other product candidate 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.

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, 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, miscellaneous selling costs, and finishing costs incurred to direct product to commercial use.

We expect that selling, general and administrative expenses will increase in future periods in connection with our efforts to commercialize EMFLAZA in the United States, and our continued efforts to commercialize Translarna for the treatment of nmDMD, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest (expense) income, net

Interest (expense) income, net consists of interest income earned on investments and interest expense from the Convertible Notes outstanding and interest expense from the Credit Facility.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with 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.

Revenue recognition

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

Net Product Sales

To date, our net product sales have consisted primarily of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. We recognize revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and we have no further performance obligations in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Subtopic 605-15, Revenue Recognition—Products.

We have recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program. Orders for Translarna are generally received from hospital and retail pharmacies and our third-party partner distributors. Our third-party distributors act as intermediaries between us and end users and do not typically stock significant quantities of Translarna. The ultimate payor for Translarna is typically a government authority or institution or a third-party health insurer. Prior to January 1, 2015, we generally recognized revenue for these reimbursed EAP programs once the product was shipped on behalf of the government authority or institution on a cash basis if all other revenue recognition criteria had

been met. Beginning in the first quarter of 2015, we are recognizing revenue for Translarna as product is shipped, as we have established a pattern of collectability.

In May 2017, EMFLAZA became commercially available in the U.S. We record product revenue related to the sales of EMFLAZA in the U.S. in accordance with ASC 605 when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured. As a result, we record net product revenue for EMFLAZA using a deferred revenue recognition model (sell-through). Under the deferred revenue model, we do not recognize revenue until EMFLAZA is shipped to an end-user. We will continue to evaluate when, if ever, we have sufficient volume of historical activity and visibility into the distribution channel, in order to reasonably make all estimates required under ASC 605 to recognize revenue upon shipment to its distributors.

We record revenue net of estimated third-party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. Allowances for government and other third-party rebates and discounts are established or estimated at the time of delivery. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

We expect that net product sales of Translarna for the treatment of nmDMD will fluctuate quarter-over-quarter. In some countries, including those in Latin America, orders for named patient sales may be for multiple months of therapy which can lead to an unevenness in orders. In addition, net product sales may fluctuate quarter-over-quarter as a result of government actions, economic pressures and political unrest. Net product sales may be significantly impacted by multiple factors, including, among other things, decisions by regulatory authorities, in particular the FDA and the EMA with respect to our submissions for Translarna for the treatment of nmDMD and our ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which we have or may obtain regulatory approval, including the United States, EEA and other territories.

Collaboration and Grant Revenue

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

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

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

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

Inventory and cost of product sales

In January 2017, the European Commission renewed our marketing authorization for Translarna for the treatment of nmDMD, subject to the specific obligation to conduct Study 041. We plan to seek to renew the marketing authorization on an annual basis until a marketing authorization that is not subject to any specific obligation is granted, if ever. A portion of the inventory available for sale was expensed as research and development costs prior to the renewal of our marketing authorization. As such,

the cost of products sold and related gross margins for the period ended June 30, 2017 are not necessarily indicative of future cost of product sales and gross margins. We expect the gross margin for Translarna to be greater than 90%, which we believe is consistent with the cost of producing small molecule therapeutics for orphan drug diseases in the pharmaceutical industry.

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. We capitalize inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Translarna and EMFLAZA product 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.

The following table summarizes the components of our inventory for the periods indicated:

	June 30, 2017	December 31, 2016
Work in progress	$ 941	$ —
Finished goods	5,971	—
Total inventory	$ 6,912	$ —

We periodically review our inventories for excess amounts or obsolescence and write down obsolete or otherwise unmarketable inventory to its estimated net realizable value. Additionally, though our product is subject to strict quality control and monitoring which we perform throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, storage costs, amortization of the acquired intangible asset and royalty payments associated with net product sales.

Accrued expenses

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

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

Share-based compensation

We expect to grant additional stock options that will result in additional share-based compensation expense. We measure the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. For service type awards, share-based compensation expense is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the entire award. For awards that vest or begin vesting upon achievement of a performance condition, we estimate the likelihood of satisfaction of the performance condition and recognize compensation expense when achievement of the performance condition is deemed probable using an accelerated attribution model.

From January 1, 2017 through June 30, 2017, we issued a total of 1,738,873 stock options to various employees. Of those, 480,550 were non-statutory stock option inducement grants made pursuant to the NASDAQ inducement grant exception as a material component of our new hires' employment compensation. All other stock option grants were made under our 2013 Long Term Incentive Plan.

The fair value of options is calculated using the Black-Scholes option pricing model to determine the fair value of stock options on the date of grant based on key assumptions, such as expected volatility and expected term. As a new public company, we do not have sufficient history to estimate the volatility of our common stock price or the expected life of the options. We calculate expected volatility based on reported data for similar publicly traded companies for which historical information is available and will continue to do so until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants.

The fair value of grants made in the six months ended June 30, 2017 was contemporaneously estimated on the date of grant using the following assumptions:

	2017
Risk-free interest rate	1.84% — 2.45%
Expected volatility	76%—81%
Expected term	5.04– 10.00 years

We assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the six month period ended June 30, 2017 was $8.07 per share.

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

Restricted Stock Awards—Restricted stock awards are granted subject to certain restrictions, including service conditions. The grant-date fair value of restricted stock awards, which has been determined based upon the market value of our common stock on the grant date, is expensed over the vesting period.

Restricted Stock Units—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 units, which has been determined based upon the market value of our common stock on the grant date, is expensed over the vesting period.

The following table summarizes information on our restricted stock awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2017	271,651	$ 19.76
Granted	358,194	$ 11.34
Vested	(180,861)	$ 14.19
Forfeited	(24,998)	$ 13.47
June 30, 2017	423,986	$ 15.39

Stock Appreciation Rights—Stock appreciation rights (SARs) entitle the holder to receive, upon exercise, an amount of our common stock or cash (or a combination thereof) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of our common stock over the measurement price based on the exercise date.

In May 2016, a total of 897,290 SARs were granted to non-executive employees (the 2016 SARs). The 2016 SARs will vest annually in equal installments over four years and will be settled in cash on each vest date, requiring us to remeasure the SARs at each reporting period until vesting occurs. For the period ending June 30, 2017, a total of 213,197 SARs vested and we recorded $1.3 million in compensation expense related to the 2016 SARs.

Employee Stock Purchase Plan—In June 2016, we established an Employee Stock Purchase Plan ("ESPP" or "the Plan") for certain eligible employees. The Plan is administered by our Board of Directors or a committee appointed by the Board. The total number of shares available for purchase under the Plan is one million shares of our common stock. Employees may participate over a six-month period through payroll withholdings and may purchase, at the end of the six-month period, our common stock at a purchase price of at least 85% of the closing price of a share of our common stock on the first business day of the offering period or the closing price of a share of our common stock on the last business day of the offering period, whichever is lower. No participant will be granted a right to purchase our common stock under the Plan if such participant would own more than 5% of the total combined voting power of us or one of our subsidiaries. For the period ending June 30, 2017, we issued 107,499 shares of common stock and recorded $0.3 million in compensation expense related to the ESPP.

We 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 June 30,		Six months ended June 30,	
	2017	2016	2016	2015
Research and development	$ 3,895	$ 4,087	$ 8,362	$ 8,415
Selling, general and administrative	3,990	4,649	8,552	9,236
Total	$ 7,885	$ 8,736	$ 16,914	$ 17,651

As of June 30, 2017, there was approximately $56.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, the 2013 Long Term Incentive Plan and equity awards made pursuant to the NASDAQ inducement grant exception for new hires. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.19 years.

Results of operations

Three months ended June 30, 2017 compared to three months ended June 30, 2016

The following table summarizes revenues and selected expense and other income data for the three months ended June 30, 2017 and 2016.

(in thousands)	Three Months Ended June 30, 2017	Three Months Ended June 30, 2016	Change 2017 vs. 2016
Net product revenue	$ 47,891	$ 15,437	$ 32,454
Collaboration and grant revenue	71	196	(125)
Cost of product sales	758	—	758
Research and development expense	30,835	28,827	2,008
Selling, general and administrative expense	28,866	23,366	5,500
Interest expense, net	(3,008)	(2,060)	(948)
Other expense, net	(1,820)	(387)	(1,433)
Income tax (expense) benefit	(150)	93	(243)

Net product revenues. Net product revenues were $47.9 million for the three months ended June 30, 2017, an increase of $32.5 million, or 210%, from $15.4 million for the three months ended June 30, 2016. The increase in net product revenue was primarily due to the increase in net product sales in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to net product sales from the domestic commercial launch of EMFLAZA in May 2017.

Collaboration and grant revenues. Collaboration and grant revenues were $0.1 million for the three months ended June 30, 2017 and $0.2 million for the three months ended June 30, 2016. Revenues are primarily from ongoing collaboration arrangements with Roche.

Cost of product sales. Cost of product sales were $0.8 million for the three months ended June 30, 2017. Cost of product sales consist primarily of amortization of the acquired intangible asset, royalty payments associated with EMFLAZA and Translarna net product sales and costs associated with EMFLAZA and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Research and development expense. Research and development expense was $30.8 million for the three months ended June 30, 2017, an increase of $2.0 million, or 7%, from $28.8 million for the three months ended June 30, 2016. The increase resulted primarily due to start-up clinical activities and regulatory spend, partially offset by the decreased costs due to the completion of our CF program at the end of 2016.

Selling, general and administrative expense. Selling, general and administrative expense was $28.9 million for the three months ended June 30, 2017, an increase of $5.5 million, or 24%, from $23.4 million for the three months ended June 30, 2016. The increase resulted primarily from the expansion of the U.S. commercial sales team in support of the domestic product launch of EMFLAZA.

Interest expense, net. Interest expense, net was $3.0 million for the three months ended June 30, 2017, an increase of $0.9 million, or 46%, from $2.1 million for the three months ended June 30, 2016. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Facility in addition to lower interest income from investments.

Other expense, net. Other expense, net was $1.8 million for the three months ended June 30, 2017, an increase in expense of $1.4 million, or 370%, from $0.4 million for the three months ended June 30, 2016. The increase resulted primarily from foreign currency losses due to changes in exchange rates in the current period.

Income tax expense. Income tax expense was $0.2 million for the three months ended June 30, 2017 and income tax benefit was $0.1 million for the three months ended June 30, 2016. We are subject to income taxes in the United States, although currently not a tax payer, and various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

The income tax expense for the three months ended June 30, 2017 differed from the amounts computed by applying the U.S. federal income tax rate of 34% to loss before tax expense as a result of the favorable amount of profit mix in foreign jurisdictions which have lower tax rates, as well as by having a full valuation allowance in jurisdictions where we have net operating losses. We review the expected annual effective income tax rate and make changes on a quarterly basis as necessary based on certain factors such as changes in forecasted annual operating income, changes to the actual and permanent book-to-tax differences, and changes resulting from the impact of tax law changes.

Six months ended June 30, 2017 compared to six months ended June 30, 2016

The following table summarizes revenues and selected expense and other income data for the six months ended June 30, 2017 and 2016.

(in thousands)	Six Months Ended June 30, 2017	Six Months Ended June 30, 2016	Change 2017 vs. 2016
Net product revenue	$ 74,334	$ 34,314	$ 40,020
Collaboration and grant revenue	176	214	(38)
Cost of product sales	797	—	797
Research and development expense	58,198	60,226	(2,028)
Selling, general and administrative expense	54,365	49,304	5,061
Interest expense, net	(5,227)	(4,016)	(1,211)
Other expense, net	(2,139)	(1,107)	(1,032)
Income tax expense	(316)	(22)	(294)

Net product revenues. Net product revenues were $74.3 million for the six months ended June 30, 2017, an increase of $40.0 million, or 117%, from $34.3 million for the six months ended June 30, 2016. The increase in net product revenue was primarily due to the increase in net product sales in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to net product sales from the domestic commercial launch of EMFLAZA in May 2017.

Collaboration and grant revenues. Collaboration and grant revenues were $0.2 million for the six months ended June 30, 2017, a decrease of $0.04 million, or 18%, from $0.2 million for the six months ended June 30, 2016. These revenues are primarily from ongoing collaboration arrangements with Roche.

Cost of product sales. Cost of product sales were $0.8 million for the six months ended June 30, 2017. Cost of product sales consist primarily of amortization of the acquired intangible asset, royalty payments associated with EMFLAZA and Translarna net product sales and costs associated with EMFLAZA and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Research and development expense. Research and development expense was $58.2 million for the six months ended June 30, 2017, a decrease of $2.0 million, or 3%, from $60.2 million for the six months ended June 30, 2016. The decrease resulted primarily from the completion of our ACT CF study at the end of 2016.

Selling, general and administrative expense. Selling, general and administrative expense was $54.4 million for the six months ended June 30, 2017, an increase of $5.1 million, or 10%, from $49.3 million for the six months ended June 30, 2016. The increase resulted primarily from the expansion of the U.S. commercial sales team in support of the domestic product launch of EMFLAZA.

Interest expense, net. Interest expense, net was $5.2 million for the six months ended June 30, 2017, an increase in expense of $1.2 million, or 30%, from interest expense of $4.0 million for the six months ended June 30, 2016. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Facility partially offset by interest income from investments.

Other expense, net. Other expense, net was $2.1 million for the six months ended June 30, 2017, an increase in expense of $1.0 million, or 93% , from other expense, net of $1.1 million for six months ended June 30, 2016. The increase resulted primarily from foreign currency losses due to changes in exchange rates in the current period.

Income tax expense. Income tax expense was $0.3 million for the six months ended June 30, 2017 and $0.0 million for the six months ended June 30, 2016. We are subject to income taxes in the United States, although currently not a tax payer, and various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

The income tax expense for the six months ended June 30, 2017 differed from the amounts computed by applying the U.S. federal income tax rate of 34% to loss before tax expense as a result of the favorable amount of profit mix in foreign jurisdictions which have lower tax rates, as well as by having a full valuation allowance in jurisdictions where we have net operating losses. We review the expected annual effective income tax rate and make changes on a quarterly basis as necessary based on certain factors such as changes in forecasted annual operating income, changes to the actual and permanent book-to-tax differences, and changes resulting from the impact of tax law changes.

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 Translarna for nmDMD and EMFLAZA for the treatment of DMD while also devoting a substantial portion of our efforts on research and development programs related to Translarna and our other product candidates.

To date, almost all of our product revenue has been attributable to sales of Translarna for the treatment of nmDMD in territories outside of 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 authorization 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. Although we have recently begun to commercialize and market EMFLAZA in the United States, to date, we have not generated significant revenue from EMFLAZA. 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.

On April 20, 2017, we completed our acquisition of all rights to EMFLAZA for total consideration comprised of $75 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock, which was determined by dividing $65.0 million by the volume weighted average price per share of our common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing. As a result of this acquisition, we expect to continue to incur additional significant costs including costs related to our efforts to commercialize EMFLAZA and satisfy related FDA post-marketing requirements.

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, the private placements of our preferred stock, collaborations, bank 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. Since 2014, we have also relied on revenues generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, we began to recognize revenue generated from net sales of EMFLAZA for the treatment of DMD in the United States. Based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future, which we anticipate will be partially offset by revenues generated from the sale of both Translarna and EMFLAZA. As a result, while we expect to continue to generate operating losses in 2017, we anticipate that operating losses generated in future periods should decline versus prior periods. The net losses we incur may fluctuate significantly from quarter to quarter.

On May 5, 2017, we entered into the Credit Agreement with MidCap Financial, which provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing 12 month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier. The facility is structured to require only monthly interest payments for the initial two years with principal amortization beginning in years three and four. The facility bears interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%, as well as additional upfront and administrative fees and expenses.

In August 2015, we closed a private offering of $150 million in aggregate principal amount of 3.00% convertible senior notes due 2022 including the exercise by the initial purchasers of an option to purchase an additional $25 million in aggregate principal amount of the Convertible Notes. The Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers' discounts and commissions and the estimated offering expenses payable by us.

Cash flows

As of June 30, 2017, we had cash, cash equivalents and marketable securities of $181.1 million.

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

	Six Months Ended June 30,	
(in thousands)	**2017**	**2016**
Cash provided by (used in):		
Operating activities	(15,582)	(66,172)
Investing activities	47,887	43,114
Financing activities	40,660	34

Net cash used in operating activities was $15.6 million for the six months ended June 30, 2017 and $66.2 million for the six months ended June 30, 2016. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash provided by investing activities was $47.9 million for the six months ended June 30, 2017 and $43.1 million for the six months ended June 30, 2016. Cash provided by investing activities was related to the sale and redemption of marketable securities to fund operations, partially offset by the cash used in the acquisition of EMFLAZA.

Net cash provided by financing activities was $40.7 million for the six months ended June 30, 2017 and $0.03 million for the six months ended June 30, 2016. Cash provided by financing activities in the current period is primarily attributable to entry into the Credit Facility with MidCap and the exercise of options and issuance of stock under the ESPP.

Funding requirements

We anticipate that our expenses will increase in connection with our commercialization efforts in the United States, the EEA and other territories, including 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 Study 041 and our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to EMFLAZA in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our cancer stem cell program. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing authorization for Translarna for other indications. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

- execute our strategy for EMFLAZA in the United States, including commercialization and integration efforts;
- satisfy contractual and regulatory obligations that we assumed through the EMFLAZA acquisition;
- are required to complete any additional clinical and non-clinical trials 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 for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;
- initiate or continue the research and development of Translarna for additional indications and of our other product candidates;
- 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 the net proceeds from our term loan facility with MidCap Financial Trust, our offering of the Convertible Notes, public offerings of common stock, marketable securities and research funding that we expect to receive under our collaborations, 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 EMFLAZA for the treatment of DMD in the United States;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for EMFLAZA for the treatment of DMD in the United States and for Translarna for the treatment of nmDMD in the EEA and other territories outside of the United States;

- our ability to maintain orphan exclusivity for, and successfully complete all FDA post-marketing requirements with respect to, EMFLAZA;
- our ability to satisfy our obligations under the terms of the Credit Agreement with MidCap Financial;
- our ability to maintain the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including 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;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, whether pursuant to the file over protest process with the FDA, or otherwise, and including, whether we will be required to perform additional clinical and non-clinical trials or complete additional analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the U.S.;
- the progress and results of our pediatric study of Translarna for the treatment of nmDMD, our open label extension clinical trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and activities under our cancer stem cell program;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for both EMFLAZA and Translarna for the treatment of nmDMD and any of our other product candidates that may receive marketing authorization or any additional indications or territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our other product candidates and Translarna in other territories or for indications other than nmDMD;
- 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 Translarna for additional indications and for our other product candidates;
- revenue received from commercial sales of Translarna or EMFLAZA, or any of our other 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, including in Germany;
- 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 acquisition of EMFLAZA; 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 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.5 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company. These costs include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Additionally, we could be forced to expend significant resources in the defense of the pending securities class action lawsuits brought against us and certain of our executives, as described under Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q.

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 or debt 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.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission rules.

Contractual obligations

During the period ended June 30, 2017, there were no material changes to our contractual obligations and commitments outside the ordinary course of business from those disclosed under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Contractual Obligations” in our Annual Report on Form 10-K for the year ended December 31, 2016, other than as disclosed below.

(in thousands)	Total	Less than 1 year	1 - 3 years	4 - 5 years	More than 5 years
Minimum royalty (1)	$ 10,271	$ 1,141	$ 4,850	$ 3,424	$ 856
Credit agreement, including interest (2)	48,588	3,140	45,448	—	—
Total contractual obligations	$ 58,859	$ 4,281	$ 50,298	$ 3,424	$ 856

(1) Under an Exclusive License and Supply Agreement ("the Faes Agreement") with Faes Farma, S.A. ("Faes"), we are required to pay royalties as a percentage of or as a fixed payment with respect to net product sales by us allocable to the EMFLAZA oral suspension product. We are required to pay Faes an annual minimum royalty during the first seven calendar years with a fixed percentage royalty during the remainder of the Faes Agreement term. The amounts above reflect the minimum required payment based on the euro to U.S. dollar exchange rate as of June 30, 2017.

(2) Under the terms of the credit agreement with MidCap, we are required to make interest only payments through April 30, 2019. Commencing on May 1, 2019 and continuing for the remaining twenty-four months of the facility, we will be required to make monthly interest payments and monthly principal payments. The principal payments are to be made based on straight-line amortization of the principal over the twenty-four month period.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended June 30, 2017, 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 Annual Report on Form 10-K for the year ended December 31, 2016.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Principal Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2017. 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 June 30, 2017, our Chief Executive Officer and Principal 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 June 30, 2017 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.

In March 2016, three purported securities class action lawsuits were commenced in the United States District Court for the District of New Jersey (one each on March 3, 10, and 11), naming as defendants the Company, our Chief Executive Officer, and our Chief Financial Officer. The lawsuits have been consolidated into one action captioned *In re PTC Therapeutics, Inc. Securities Litigation*, No. 16-1224 (KM). A consolidated amended complaint was filed on January 13, 2017. On February 14, 2017, the defendants filed a motion to dismiss the consolidated amended complaint. The action alleges violations of Sections 10(b) and 20(a) and Rule 10b-5 of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company about its business, operations, and prospects as it relates to the NDA for Translarna for the treatment of nmDMD that the Company submitted to the FDA in December 2015. The plaintiffs seek, among other things, compensatory damages for purchasers of the Company's common stock between November 6, 2014 and February 23, 2016, as well as attorneys' fees and costs.

Item 1A. Risk Factors

The following risk factors and other information included in this Quarterly Report on Form 10-Q should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see page 1 of this Quarterly Report on Form 10-Q for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Recent Acquisition of EMFLAZA™ (deflazacort)

We may fail to realize the anticipated benefits of our acquisition of all rights to EMFLAZA, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.

On April 20, 2017, we completed the acquisition of all rights to EMFLAZA pursuant to an asset purchase agreement, dated March 15, 2017 and amended on April 20, 2017, or the Asset Purchase Agreement, by and between us and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon.

Our ability to realize the anticipated benefits of our acquisition of EMFLAZA will depend, to a large extent, on our ability to integrate EMFLAZA into our business and realize anticipated growth opportunities and synergies. While we recently commenced the initial phases of the commercialization launch for EMFLAZA in the United States, we have no history of commercializing pharmaceutical products in the United States, we are still in the process of completing the full commercialization of EMFLAZA in the United States and we expect the ongoing process will be complex, costly and time-consuming. As a result, we will be required to devote significant management attention and resources to integrating this product into our business. The process may be disruptive to our business and the expected benefits may not be achieved within the anticipated time frame, or at all. The failure to meet the challenges involved and to realize the anticipated benefits of the acquisition could cause an interruption of, or a loss of momentum in, our commercialization efforts and could adversely affect our business, financial condition and results of operations.

While we recently commenced the initial phases of the commercialization launch for EMFLAZA in the United States, we are still in the process of completing the full commercialization of EMFLAZA. Delays in our ability to complete the full commercialization of EMFLAZA in the United States could result in negative reactions from our stockholders, patients, the medical community, vendors, payors, and employees, among others. Further, such delays could result in declines in the price of our common stock and the perception of the effectiveness of our management and our company may suffer in the marketplace.

In addition, to the factors above relating to the commercial launch of EMFLAZA in the United States, we expect our commercialization efforts to rely on non-patent market exclusivity periods under the Orphan Drug Act of 1983, or the Orphan Drug Act, and the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. There are limited circumstances under each of the Orphan Drug Act and the Hatch-Waxman Act that could result in our loss of marketing exclusivity, which could allow a competitor to enter the market. Failure to maintain either market exclusivity period would have a material adverse effect on our ability to commercialize EMFLAZA.

While we have commenced our launch of EMFLAZA on a commercial basis in the United States, there may be delays in the processing and adjudication of prescriptions, which can require a significant period of time, in some cases several months, and may be subject to additional third-party payor requirements, including prior authorization and requirements to try other therapies first, which would delay our ability to obtain payment for prescriptions for EMFLAZA. Further, while we estimate that there are approximately 9,000 DMD patients in the United States who are aged five years or older, these estimates may prove to be incorrect. If the market opportunity for EMFLAZA is smaller than we believe it is, our business and anticipated

revenues will be negatively impacted. There are no guarantees that EMFLAZA will be commercially successful and we may never achieve significant revenue from sales of the drug.

Our ability to realize the anticipated benefits of the acquisition is expected to entail numerous additional material potential difficulties, including, among others:

- challenges related to public and market perception of EMFLAZA and/or our acquisition of the product;
- increased scrutiny from third parties, including regulators, legislative bodies and enforcement agencies, with respect to product pricing and commercialization matters;
- changes in laws or regulations that adversely impact the anticipated benefits of the acquisition;
- challenges related to the perception by patients, the medical community and third-party payors of EMFLAZA for the treatment of DMD;
- challenges related to the ability of patients to obtain and maintain sufficient coverage and reimbursement from third-party payors, including Medicare and Medicaid and other government and private payers for EMFLAZA;
- disruptions to our manufacturing arrangements with third-party manufacturers, including our exclusive providers of tablet and suspension EMFLAZA product;
- disruptions to our third-party distribution channel;
- difficulties in managing the expanded operations of a significantly larger and more complex company following the acquisition;
- the diversion of management attention to integration matters;
- difficulties in achieving anticipated business opportunities and growth prospects from the acquisition;
- the size of the treatable patient population may be smaller than we believe it is;
- difficulties in assimilating employees and in attracting and retaining key personnel; and
- potential unknown liabilities, adverse consequences, unforeseen increased expenses or other unanticipated problems associated with the acquisition.

Many of these factors are outside of our control, and any one of them could result in increased costs, decreased expected revenues and further diversion of management time and energy, which could materially impact our business, financial condition and results of operations.

In addition, we now possess not only the rights to EMFLAZA, but also certain corresponding liabilities and obligations, including the contractual liabilities and regulatory obligations that were assumed by us upon closing of the transaction, including certain royalty payment obligations and FDA post-marketing requirements. The post-marketing requirements we are obligated to complete in connection with our marketing authorization granted by the FDA in the United States are expected to result in additional investment in EMFLAZA by us, and failure to satisfy any such requirements could delay our realization of, or prevent us from ever realizing, the anticipated benefits from the acquisition.

In addition, upfront consideration for the acquisition from Marathon was comprised of $75 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock. Marathon is also entitled to receive contingent payments from us based on annual net sales of EMFLAZA beginning in 2018 and has the opportunity to receive a single $50 million sales-based milestone. The issuance of our common stock was dilutive to our existing stockholders and because we have limited financial resources, by investing in this acquisition, we may forgo or delay pursuit of other opportunities that may have proven to have greater commercial potential.

Further, it is possible that undisclosed, contingent, or other liabilities or problems may arise in the future of which we were previously unaware. These undisclosed liabilities could have an adverse effect on our business, financial condition and results of operations.

All of these factors could decrease or delay the expected accretive effect of the transaction and negatively impact our stock price. As a result, it cannot be assured that the acquisition will result in the full realization of the benefits anticipated from the transaction within the anticipated time frames or at all.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception and based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future. We may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. As of June 30, 2017, we had an accumulated deficit of $781.6 million. We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, the private placements of our preferred stock, collaborations, bank 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 and product candidates. Since 2014, we have also relied on revenues generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, we began to recognize revenue generated from net sales of EMFLAZA for the treatment of DMD in the United States. Based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future, which we anticipate will be partially offset by revenues generated from the sale of both Translarna and EMFLAZA. We expect to continue to generate operating losses in 2017 and, while we anticipate that operating losses generated in future periods should decline versus prior periods, we may never generate profits from operations or maintain profitability. The net losses we incur may fluctuate significantly from quarter to quarter.

On April 20, 2017, we acquired all rights to EMFLAZA. Upfront consideration for the acquisition was comprised of $75 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock. In addition, we expect to incur significant costs in connection with liabilities we assumed as part of the acquisition, including the obligation to complete certain post-marketing requirements in connection with the EMFLAZA marketing authorization.

On March 2, 2017, we announced that the primary and secondary endpoints were not achieved in ACT CF, our Phase 3 clinical for Translarna in nonsense mutation cystic fibrosis, or nmCF, and that, as a result, we have discontinued our current clinical development of Translarna for nmCF and have withdrawn our type II variation submission with the European Medicines Agency, or EMA, which sought approval of Translarna for the treatment of nmCF in the European Economic Area, or EEA.

In October 2015, we announced that the primary efficacy endpoint in the intent to treat, or ITT, population did not achieve statistical significance in ACT DMD, our Phase 3 clinical trial for Translarna for the treatment of nmDMD.

Our current ability to generate revenue from sales of Translarna is dependent upon our ability to maintain our marketing authorization in the EEA of Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. The marketing authorization in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further subject to a specific obligation to conduct and report the results of Study 041, a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna in the approved patient population. Enrolling, conducting and reporting a clinical trial is a time-consuming, expensive and uncertain process that takes years to complete, and we expect that we will incur material costs related to the implementation and conduct of Study 041. In addition, it is likely that we will enroll patients in Study 041 in countries where Translarna for the treatment of nmDMD is currently available on a reimbursed basis, which could negatively impact growth in our net product sales. We may experience unknown complications with Study 041 and may not achieve the pre-specified endpoint with statistical significance, which would have a material adverse effect on our ability to maintain our marketing authorization in the EEA.

If, in any annual renewal cycle, the EMA determines that the balance of benefits and risks of using Translarna for the treatment of nmDMD has changed materially or that we have not or are unable to comply with the specific obligation to complete Study 041 or any other requirement that has been or may be placed on the marketing authorization, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or impose other specific obligations or restrictions, which would have a materially adverse effect on our business. We expect to incur significant costs in connection with our efforts to maintain our marketing authorization in the EEA. If our marketing authorization in the EEA is not renewed, or our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or a reimbursed early access program, or EAP program, and throughout all territories. For additional information, see the risk factor under "Risks Related to Regulatory Approval of our Products and our Product Candidates" titled, "*Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon our ability to satisfy the specific obligation to conduct and report the results of Study 041 by September 2021, and, as such, there is ongoing risk that we may be unable to maintain such authorization. If we are unable to obtain renewal of such marketing authorization in any future renewal cycle, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, financial performance and results of operations."*

We also expect that our efforts to advance Translarna for the treatment of nmDMD in the United States, whether pursuant to the file over protest process with the FDA, or otherwise, will be time-consuming and may be expensive. For additional information, see the risk factor under "Risks Related to Regulatory Approval of our Products and our Product Candidates" titled, "*There is substantial risk that the FDA will continue to disagree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD and we will be unable to advance Translarna for the treatment of nmDMD in the United States in a timely manner, or at all, whether pursuant to the file over protest process or otherwise, and by determining to file our NDA over protest, we have postponed other available strategic pathways which may have proven to be more effective. If there are delays in obtaining regulatory approval in the United States, we will not be able to commercialize Translarna for nmDMD in that territory and our ability to generate revenue will be materially impaired. In the event that the FDA requires us to conduct a new clinical trial in nmDMD which, if successful, may enable FDA review of an NDA submission by us, we would expect to incur significant costs, which may have a material adverse effect on our business and results of operations."*

We anticipate that our expenses will further increase in connection with our commercialization efforts in the United States for EMFLAZA and in the EEA and other territories for Translarna for the treatment of nmDMD, 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, the clinical and regulatory developments noted in this risk factor may exacerbate the risks related to our commercialization efforts set forth under the heading "Risks Related to the Development and Commercialization of our Products and our Product Candidates," which could increase the costs associated with our commercial activities or have a negative impact on our revenues. For additional information, see also, the risk factor under the heading "Risks Related to the Regulation of our Products and our Product Candidates" titled "*Commercialization of Translarna has been in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna for the treatment of nmDMD in the EEA and other countries where Translarna is available would delay or prevent us from marketing our product in such regions, which would adversely affect our anticipated revenue, growth and business.*"

We may seek to further expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies, similar to our recent acquisition of EMFLAZA, 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.

In addition to the foregoing, we expect to continue to incur significant costs in connection with our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to EMFLAZA in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our cancer stem cell program.

We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA. These efforts may significantly impact the timing and extent of our commercialization expenses.

With respect to our outstanding 3.00% convertible senior notes due August 15, 2022, or the Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.5 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears.

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

- execute our strategy for EMFLAZA in the United States, including commercialization and integration efforts;
- satisfy contractual and regulatory obligations that we assumed through the EMFLAZA acquisition;
- are required to complete any additional clinical and non-clinical trials 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 for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;
- initiate or continue the research and development of Translarna for additional indications and of our other product candidates;
- 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 also could be forced to expend significant resources in the defense of the pending securities class action lawsuits brought against us and certain of our executives, as described under Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q.

Our ability to generate profits from operations and become and remain profitable depends on our ability to successfully develop and commercialize drugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including:

- completing our ongoing launch of commercial sales of EMFLAZA for the treatment of DMD in the United States in accordance with our estimated timeline;
- negotiating, securing, and maintaining adequate pricing, coverage and reimbursement terms, on a timely basis, for EMFLAZA for the treatment of DMD in the United States;
- maintaining orphan exclusivity for EMFLAZA and successfully completing all FDA post-marketing requirements with respect to EMFLAZA;
- maintaining the marketing authorization of Translarna for the treatment of nmDMD in the EEA, including successfully obtaining annual renewals of the marketing authorization, fulfilling the specific obligation to conduct and report the results of Study 041 to the EMA, and meeting any ongoing requirements related to the marketing authorization;
- advancing Translarna for the treatment of nmDMD in the United States in a timely manner, or at all, whether pursuant to the file over protest process with the FDA or otherwise, and including, if required, performing additional clinical and non-clinical trials or analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- expanding the territories in which we are approved to market Translarna for the treatment of nmDMD;
- minimizing the enrollment impact of Study 041 on commercialization efforts for Translarna for nmDMD;
- developing Translarna for the treatment of additional indications, including nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and successfully advancing our other programs and collaborations, including our cancer stem cell and SMA programs;
- establishing a global commercial infrastructure, including the sales, marketing and distribution capabilities to effectively market and sell Translarna for the treatment of nmDMD in the EEA and other parts of the world;
- implementing marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- negotiating, securing and maintaining adequate pricing and reimbursement terms for Translarna for the treatment of nmDMD on a timely basis, or at all, in the countries in which we have obtained, and may obtain, regulatory approval;
- launching commercial sales of Translarna for the treatment of nmDMD in accordance with our estimated timeline;
- identifying patients eligible for treatment with EMFLAZA for DMD;
- identifying patients eligible for treatment with Translarna for nmDMD;
- obtaining approval to market Translarna for the treatment of other indications;
- expanding the approved product label of Translarna for the treatment of nmDMD;
- successfully developing or commercializing any product candidate or product that we may in-license or acquire;
- protecting our rights to our intellectual property portfolio related to Translarna; and

- contracting for the manufacture and distribution of commercial quantities of Translarna and EMFLAZA.

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

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

As noted in the prior risk factor, we expect to incur significant expenses related to our clinical, regulatory, commercial, legal, research and development, and other business efforts. We believe that our cash flows from product sales, together with existing cash and cash equivalents, including the net proceeds from our term loan facility with MidCap Financial, our Convertible Note offering, public offerings of common stock, marketable securities and research funding that we expect to receive under our collaborations, 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 EMFLAZA for the treatment of DMD in the United States;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, for EMFLAZA for the treatment of DMD in the United States and Translarna for the treatment of nmDMD in the EEA and other territories outside of the United States;
- our ability to maintain orphan exclusivity for, and successfully complete all FDA post-marketing requirements with respect to, EMFLAZA;
- our ability to satisfy our obligations under the terms of the credit and security agreement with MidCap Financial;
- our ability to maintain the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including 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;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, whether pursuant to the file over protest process with the FDA, or otherwise, and including, whether we will be required to perform additional clinical and non-clinical trials or complete additional analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the U.S.;
- the progress and results of our pediatric study of Translarna for the treatment of nmDMD, our open label extension clinical trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and activities under our cancer stem cell program;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for both EMFLAZA for the treatment of DMD and Translarna for the treatment of nmDMD and any of our other product candidates that may receive marketing authorization or any additional indications or territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our other product candidates and Translarna in other territories or for indications other than nmDMD;
- 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 Translarna for additional indications and for our other product candidates;
- revenue received from commercial sales of Translarna or EMFLAZA or any of our other 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, including in Germany;

- 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 acquisition of EMFLAZA; 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.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales for certain product candidates or indications. In addition, our products and product candidates, if approved, may not achieve commercial success, including Translarna for the treatment of nmDMD and EMFLAZA for the treatment of DMD.

To date almost all of our product revenue has been attributable to sales of Translarna for the treatment of nmDMD in territories outside of the United States. We are continuing to engage in significant commercialization efforts for this product. In order to continue sales and our commercial launch of Translarna, we must maintain our marketing authorization 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. Although we have recently begun to commercialize and market EMFLAZA in the United States, to date, we have not generated significant revenue from EMFLAZA. 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.

Other commercial revenue, if any, would be derived from product acquisitions or, if none, from sales of products that we are not planning to have commercially available for several years, if at all. If our marketing authorization for Translarna in the EEA is not renewed, or our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna for the treatment of nmDMD, whether pursuant to a commercial or an EAP program and throughout all territories. Likewise, if we fail to maintain our marketing authorization for EMFLAZA in the United States or lose the non-patent market exclusivity for EMFLAZA, we will be unable to commercialize and generate revenue from sales of that product.

Accordingly, we will need to continue to rely on additional financing in connection with our continuing operations and to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or based on strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms or at all. 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.

Our indebtedness resulting from our credit and security agreement with MidCap Financial Trust could adversely affect our financial condition or restrict our future operations.

On May 5, 2017, we entered into a credit and security agreement with Midcap Financial Trust, a Delaware statutory trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto, or the Credit Agreement, that provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing twelve month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%. The Credit Agreement requires us to not have less than $100 million of net revenue (raised to $120 million if the additional $20 million term loan is issued) for the prior twelve months to be measured on the last day of each fiscal quarter beginning on December 31, 2017.

Additionally, subject to customary exceptions and exclusions, all obligations under the Credit Agreement are secured pursuant to the terms of the Credit Agreement, a Pledge Agreement between us, certain of our subsidiaries, and Midcap Financial, or the Pledge Agreement, and an Intellectual Property and Security Agreement between us and Midcap Financial, or the IP Security Agreement, each dated May 5, 2017. Under the Credit Agreement, the Pledge Agreement, and the IP Security Agreement, we provided to Midcap Financial and the other lenders a perfected, first-priority security interest in all of our personal property, a perfected, first-priority security interest in all of our intellectual property (except that this security interest will not be perfected in intellectual property located outside the United States unless our cash position falls below a pre-specified threshold), and a perfected, first-priority pledge of 65% of the equity ownership interests directly held by us in our wholly owned subsidiary, PTC Therapeutics Holdings (Bermuda) Corp. Limited.

A failure to comply with the conditions of our Credit Agreement could result in an event of default. An event of default under the Credit Agreement includes, among other things, a failure to pay any amount due under the Credit Agreement as well as the occurrence of events that could reasonably be expected to result in a material adverse effect, including if we were to fail to maintain our marketing authorization for Translarna for the treatment of nmDMD in the EEA or if the FDA were to withdraw any of our products from the market, including EMFLAZA for the treatment of DMD in the United States.

In the event of an acceleration of amounts due under our Credit Agreement as a result of an event of default, we may not have sufficient funds or may be unable to arrange for additional financing to repay the term loans or to make any accelerated payments, and the lenders could seek to enforce security interests in the collateral securing the term loans, which would have a material adverse effect on our business, financial condition and results of operations.

In addition, our indebtedness under the Credit Agreement could have significant adverse consequences, including, among other things:

- requiring us to dedicate a substantial portion of cash and cash equivalents and marketable securities to the payment of interest on, and principal of, the term loans, which will reduce the amounts available to fund working capital, capital expenditures, product development efforts and other general corporate purposes;
- obligating us to negative covenants restricting our activities, including limitations on dispositions, mergers or acquisitions, encumbering our intellectual property, incurring indebtedness or liens, paying dividends, making investments and engaging in certain other business transactions;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates; and
- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes.

Any of these factors could materially and adversely affect our business, financial condition and results of operations.

We may engage in strategic transactions to acquire assets, businesses, or rights to products, product candidates or technologies or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, increase our debt, or cause us to incur significant expense.

As part of our business strategy, we may engage in additional strategic transactions to expand and diversify our product pipeline, including through the acquisition of assets, businesses, or rights to products, product candidates or technologies or through strategic alliances or collaborations, similar to our recent acquisition of EMFLAZA. We may not identify suitable strategic transactions, or complete such transactions in a timely manner, on a cost-effective basis, or at all. Moreover, we may devote resources to potential opportunities that are never completed or we may incorrectly judge the value or worth of such opportunities. Even if we successfully execute a strategic transaction, we may not be able to realize the anticipated benefits of such transaction, may incur additional debt or assume unknown or contingent liabilities in connection therewith, and may experience losses related to our investments in such transactions. Integration of an acquired company or assets into our existing business may not be successful and may disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, and require management resources that would otherwise focus on developing our existing business. Even if we are able to achieve the long-term benefits of a strategic transaction, our expenses and short-term costs may increase materially and adversely affect our liquidity. Any of the foregoing could have a detrimental effect on our business, results of operations and financial condition.

In addition, future strategic transactions may entail numerous operational, financial and legal risks, including:

- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- exposure to known and unknown liabilities, including possible intellectual property infringement claims, violations of laws, tax liabilities and commercial disputes;
- higher than expected acquisition and integration costs;

- difficulty in integrating operations and personnel of any acquired business;
- increased amortization expenses or, in the event that we write-down the value of acquired assets, impairment losses;
- impairment of relationships with key suppliers or customers of any acquired business due to changes in management and ownership;
- inability to retain personnel, customers, distributors, vendors and other business partners integral to an in-licensed or acquired product, product candidate or technology;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges;
- entry into indications or markets in which we have no or limited direct prior development or commercial experience and where competitors in such markets have stronger market positions; and
- other challenges associated with managing an increasingly diversified business.

If we are unable to successfully manage any strategic transaction in which we may engage, our ability to develop new products and continue to expand and diversify our product pipeline may be limited.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

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

To the extent that we raise additional capital through the sale of equity or convertible debt securities, 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, entering into agreements involving licenses to our intellectual property, 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 grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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

Since mid-2014, we have been transitioning from a company with a research and development focus to a company capable of supporting global commercial activities. We may not be successful in completing this transition. Our ability to develop product candidates, manufacture a commercial scale product or arrange for a third party to do so on our behalf, and conduct sales and marketing activities necessary for a successful full scale product commercialization is largely limited to our activities with respect to the marketing authorization in the EEA for Translarna for the treatment of nmDMD, which is subject to annual review and renewal following reassessment of the benefit-risk balance of the authorization by the EMA and satisfaction of the specific obligation to conduct and report to the EMA Study 041. In addition, other than our marketing authorization in the EEA and the marketing authorizations granted in Israel and South Korea (which are largely contingent upon continued EMA approval), we have not proven our ability to successfully obtain marketing authorizations to sell our products or product candidates. Although we recently commenced the initial phases of the commercialization launch for EMFLAZA, we have no history of commercializing pharmaceutical products in the United States, we are still in the process of completing the full commercialization of EMFLAZA and, to date, we have not generated significant revenue from EMFLAZA in the United States. Further, we recently announced that we are discontinuing our current clinical development of Translarna for nmCF based on the results of ACT CF, and we may not successfully complete development of other product candidates. Consequently, any predictions our stockholders make about our future success or viability may not be as accurate as they could be if we had a longer operating history. In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors, including, and not limited to, those related to our commercialization of EMFLAZA and integration of EMFLAZA into our business.

Our ability to use our net operating losses and certain other tax attributes to offset potential taxable income and related income taxes that would otherwise be due is subject to limitation under the provisions of Sections 382 and 383 of the Code as a result of ownership changes of the Company and could be subject to further annual limitations under such provisions.

In addition, we may not generate sufficient future taxable income to use our net operating losses and certain other tax attributes.

If a corporation undergoes an "ownership change" within the meaning of Sections 382 and 383 of the Internal Revenue Code, or Sections 382 and 383, the corporation's ability to utilize any net operating losses, or NOLs, and certain tax credits and other attributes generated before such an ownership change, is limited. We believe that we have in the past experienced ownership changes within the meaning of Sections 382 and 383 that have resulted in limitations under Sections 382 and 383 (and similar state provisions) on the use of our NOLs and other tax attributes.

However, Sections 382 and 383 of the Code are extremely complex provisions with respect to which there are many uncertainties, and we have not requested a ruling from the IRS to confirm our analysis of the ownership change limitations related to the NOLs generated by the Company. Therefore, we have not established whether the IRS would agree with our analysis regarding the application of Section 382 and 383 of the Code. If the IRS were to disagree with our analysis, or if the Company experiences additional ownership changes in the future, such as the recent issue of shares to Marathon, we could be subject to further annual limitations on the use of the NOLs to offset potential taxable income and related income taxes that would otherwise be due.

Moreover, our ability to use these NOLs to offset potential future taxable income and related income taxes that would otherwise be due is dependent upon our generation of future taxable income before the expiration dates of the NOLs, and we cannot predict with certainty when, or whether the Company will generate sufficient taxable income to use all of the NOLs.

Changes in our effective income tax rates and future changes to U.S. and non-U.S. tax laws could adversely affect our results of operations.

We are subject to income taxes in the Unites States and various foreign jurisdictions. Taxes will be incurred as income is earned among these different jurisdictions. Various factors may have favorable or unfavorable effects on our effective income tax rate. These factors include, but are not limited to, interpretations of existing tax laws, changes in tax laws and rates, the accounting for stock options and other share-based compensation, changes in accounting standards, future levels of research and development spending, changes in the mix and level of pre-tax earnings by taxing jurisdiction, the outcome of examinations by the U.S. Internal Revenue Service and other jurisdictions, the accuracy of our estimates for unrecognized tax benefits, the realization of deferred tax assets, or by changes to our ownership or capital structure. The impact on our income tax provision resulting from the above-mentioned factors and others may be significant and could adversely affect our results of operations.

In addition, there is growing pressure in many jurisdictions (including the United States) and from multinational organizations such as the Organization for Economic Co-operation and Development, or OECD, and the EU, to amend existing international tax rules in order to render them more responsive to current global business practices. For example, the OECD has released guidance relating to various international tax related topics in an initiative referred to as Base Erosion and Profit Shifting, or BEPS, that aims to standardize and modernize global tax policy. Legislation to adopt these standards has been enacted or is currently under consideration in a number of jurisdictions. As a result, BEPS could have material adverse consequences on our effective tax rate, the amount of tax we pay and on our financial position and results of operations.

In addition, the new Presidential Administration and U.S. Congress have called for comprehensive tax reform and most recently, President Trump has released an outline of a proposed tax plan which would significantly alter the U.S. tax code if enacted. These proposals include, among other items, a significant reduction to the U.S. corporate tax rate and a possible "border adjustment tax" that would effectively increase the economic cost of imports.

Although we monitor these developments, it is very difficult to assess to what extent these changes may be implemented in the United States and other jurisdictions in which we conduct our business or may impact the way in which we conduct our business or our effective tax rate due to the unpredictability and interdependency of these potential changes. Changes in tax laws and related regulations and practices could have a material adverse effect on our business operations, cash flows, effective tax rate, financial position and results of operations.

Risks Related to the Development and Commercialization of our Products and our Product Candidates

ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations.

In October 2015, we announced that the primary efficacy endpoint in the ITT population did not achieve statistical significance in ACT DMD. On the basis of our position that the totality of clinical data from ACT DMD and our prior Phase 2b trial support the clinical benefit of Translarna for the treatment of nmDMD, we submitted our analyses of the ACT DMD data and meta-analysis of the combined ACT DMD and Phase 2b subgroup data to the FDA, as part of our NDA.

On February 22, 2016, we received a Refuse to File letter from the FDA stating that, in the view of the FDA, both our Phase 2b and Phase 3 ACT DMD trials were negative and do not provide substantial evidence of effectiveness. Additionally, the FDA stated that we had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. In addition, the FDA noted that our NDA does not contain adequate information regarding the abuse potential of Translarna. In October 2016, the FDA denied our first appeal of the Refuse to File letter. In the first quarter of 2017, we filed our Translarna NDA for nmDMD with the FDA via the "file over protest" process that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission. When an application is filed over protest, the FDA is required to review the application as filed. Generally, the FDA does not favor the file over protest procedure and the agency's policies explain that an application filed over protest does not receive a timeline for review and is designated as a standard review. The FDA has granted a standard review for the Translarna NDA and has set a target review date under the Prescription Drug User Fee Act, or PDUFA, of October 24, 2017, and has tentatively scheduled an advisory committee meeting on September 28, 2017 to facilitate its review. The PDUFA date is the goal date for the FDA to complete its review of the NDA, however, such date is not binding on the agency and there can be no assurance that the FDA will complete its review of our NDA by the PDUFA goal date.

There is substantial risk that, notwithstanding any dialogue we have had or any further dialogue we may be able to initiate with the agency, pursuant to the file over protest process or otherwise, the FDA will continue to disagree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials. Even if we are successful in resolving some or all of the matters raised by the FDA in the Refuse to File letter, there is significant risk that we will be unable to obtain FDA approval of Translarna for nmDMD, on a timely basis or at all, and we may be required to perform additional clinical and non-clinical trials or complete additional analyses at significant cost. Even if we are able to enroll and fund any such additional trials or complete such analyses, there is substantial risk that the results would not ultimately support the approval of the NDA filed over protest or a new NDA submission in the United States for Translarna for nmDMD. In addition, any such requirement for additional trials would most likely result in our inability to sell Translarna in the United States for a significant period of time, which would have a material adverse effect on our ability to generate revenue from the sales of Translarna for the treatment of nmDMD. Due to these uncertainties, we are unable to estimate the timing or potential for a launch of Translarna for the treatment of nmDMD in the United States.

We also submitted our analyses of the ACT DMD data and meta-analyses of the combined ACT DMD and Phase 2b subgroup data to the EMA to support continuation of our marketing authorization in the EEA, which is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization. The EMA and European Commission did not approve our request for full marketing authorization of Translarna for the treatment of nmDMD and, instead, approved the annual renewal of our conditional marketing authorization with the specific obligation to confirm the efficacy and safety of Translarna for the treatment of nmDMD in ambulatory patients aged 5 years or older via Study 041.

Enrolling, conducting and reporting a clinical trial is a time-consuming, expensive and uncertain process that takes years to complete, and we expect that we will incur material costs related to the implementation and conduct of Study 041. We expect that conducting a placebo-controlled trial in nmDMD of this size will be challenging and it is probable that we will enroll patients in territories where Translarna has already become available on a reimbursed basis, which could negatively impact growth in our product sales. We may enroll patients in countries with a different standard of care for nmDMD patients or at clinical trial sites that are inexperienced with clinical trials in general, or specifically with nmDMD trials. In addition, we may experience unknown complications with Study 041 and may not achieve the pre-specified endpoint with statistical significance, which would have a material adverse effect on our ability to maintain our marketing authorization in the EEA.

The marketing authorization renewal approved in June 2017 is effective through August 5, 2018, unless extended. If the EMA determines in any annual renewal cycle that the balance of benefits and risks of using Translarna for the treatment of nmDMD has changed materially or that we have not or are unable to comply with any conditions that have been or may be placed on the marketing authorization, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require the imposition of other conditions or restrictions. As such, there is ongoing risk to our ability to maintain our marketing authorization in the EEA.

Our current ability to generate revenue from sales of Translarna is dependent upon our ability to maintain our marketing authorization in the EEA of Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. If we are unable to renew our EEA marketing authorization during any annual renewal cycle, or if our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program, which would have a material adverse effect on our business, results of operations and financial condition.

There is substantial risk that other regulators in regions where we have not yet sought or are currently seeking marketing authorization will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in

Translarna for the treatment of nmDMD, which would have a material adverse effect on our ability to generate revenue from the sales of Translarna for the treatment of nmDMD in those applicable territories. In addition, we may not be able to maintain or obtain marketing authorizations in areas where such authorizations are contingent upon decisions of the EMA with respect to our marketing authorization in the EEA.

For additional information, see "Risks Related to Regulatory Approval of our Products and our Product Candidates" below.

We depend heavily on the success of Translarna, which we are developing for nmDMD and other indications, and EMFLAZA, for DMD. If we are unable to execute our commercial strategy for Translarna for the treatment of nmDMD in the EEA or for EMFLAZA for the treatment of DMD in the United States, fail to receive regulatory approval for Translarna for the treatment of nmDMD in the United States and other territories, fail to obtain renewal of, or satisfy the conditions of our marketing authorization for Translarna for the treatment of nmDMD in the EEA, or fail to maintain our marketing authorization or market exclusivity for EMFLAZA in the United States, or if we experience significant delays in accomplishing such goals, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the development of Translarna for nmDMD, and most recently, in the acquisition of EMFLAZA for DMD. Our ability to generate product revenues will depend heavily on the successful development and commercialization of Translarna and our successful integration of EMFLAZA into our business and commercialization of EMFLAZA for DMD in the United States.

We have no history of commercializing pharmaceutical products in the United States. While we recently commenced the initial phases of the commercialization launch for EMFLAZA in the United States, we are still in the process of completing the full commercialization of EMFLAZA and there can be no assurances that we will be successful in this endeavor. As we presently have no patent rights to protect the approved use of EMFLAZA, we expect to rely on the concurrently running market exclusivity periods currently available to us under the Hatch-Waxman Act and the Orphan Drug Act to commercialize EMFLAZA for DMD in the United States. Further, we are obligated to complete certain FDA post-marketing requirements in connection with our marketing authorization of EMFLAZA. Failure to maintain these market exclusivity periods, complete the FDA post-marketing requirements, maintain our marketing authorization for EMFLAZA in the United States, or timely execute our commercialization plans for EMFLAZA, would have a material adverse effect on our business, financial position and results of operations.

While we have obtained marketing authorization for Translarna for the treatment of nmDMD in the EEA, such authorization is subject to annual review and renewal by the European Commission following the annual EMA reassessment as well as the specific obligation to conduct and submit the results of Study 041. For a review of recent developments that have had, and may continue to have, a material adverse effect on our ability to commercialize Translarna for the treatment of nmDMD, please review the risk factor titled, "*ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations.*"

We are currently pursuing further clinical development efforts for Translarna for the treatment of nmDMD, nonsense mutation aniridia, and nonsense mutation CDKL5/Dravet syndrome. Each genetic disorder has unique genetic and pathophysiological characteristics and we believe that regulators, including the FDA and the EMA, will evaluate the effectiveness of Translarna for any given indication based on the merits of the clinical efficacy evidence available for such indication. However, because we are developing Translarna for the treatment of multiple indications associated with genetic disorders that arise as a result of a nonsense mutation, there is a risk that negative results in a clinical trial evaluating the efficacy of Translarna for one indication, such as ACT CF, could adversely affect the perception of the efficacy of Translarna in a different indication. There can be no assurance that regulators, including the FDA and the EMA, will not consider such results when making determinations with respect to our ongoing or future regulatory submissions for marketing authorization of Translarna for any indication, including in connection with the FDA's review of our NDA (which was filed over protest with the FDA in the first quarter of 2017) for Translarna for the treatment of nmDMD and the EMA's annual reassessment of our marketing authorization for Translarna for the treatment of nmDMD, which could have an adverse effect on the outcome of the applicable regulatory review. We intend to submit the safety results of ACT CF to regulators with any applicable safety updates and submissions, including the EMA. While the safety profile of Translarna in the ACT CF study was consistent with previous studies and no new safety signals were identified, there can be no assurance that the EMA or other regulators will agree with our interpretation of the safety data from the trial.

If we do not successfully renew and maintain our marketing authorization and commercialize Translarna in the EEA, or receive regulatory approval in the United States for Translarna for the treatment of nmDMD and subsequently successfully commercialize Translarna in the United States, our ability to generate additional revenue will be jeopardized and, consequently, our business will be materially harmed. Likewise, if we do not successfully commercialize or maintain our marketing

authorization for EMFLAZA for the treatment of DMD in the United States, our ability to generate additional revenue will be jeopardized and, consequently, our business will be materially harmed.

The success of EMFLAZA and Translarna will depend on a number of additional factors, including the following:

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms on a timely basis, or at all;
- the timing and scope of commercial launches;
- the maintenance and expansion of a commercial infrastructure capable of supporting product sales, marketing and distribution;
- the implementation and maintenance of marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- our ability to establish and maintain commercial manufacturing arrangements with third-party manufacturers;
- the ability of our third-party manufacturers to successfully produce commercial and clinical supply of drug on a timely basis sufficient to meet the needs of our commercial and clinical activities;
- successful identification of eligible patients;
- acceptance of the drug as a treatment for the approved indication by patients, the medical community and third-party payors, including, with respect to Translarna any impact the results of ACT CF may have on the perception of the effectiveness of Translarna;
- effectively competing with other therapies;
- a continued acceptable safety profile of the drug;
- the costs, timing and outcome of post-marketing studies and trials for EMFLAZA and Translarna, including, with respect to Translarna, Study 041;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity, including with respect to EMFLAZA, whether we are able to maintain market exclusivity periods under the Hatch-Waxman Act and Orphan Drug Act; and
- whether, with respect to Translarna, we are able to continue to satisfy our obligations under, and maintain, the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including 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;
- whether, and within what timeframe, we are able to advance Translarna for the treatment of nmDMD in the United States, pursuant to the file over protest process with the FDA or otherwise, and including, whether we will be required to perform additional clinical and non-clinical trials or complete additional analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- the successful advancement of Translarna in additional indications, in particular, nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5;
- 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;
- our ability to successfully prepare and advance regulatory submissions for marketing authorizations for Translarna in additional territories and for additional or expanded indications and whether and in what timeframe we may obtain such authorizations;
- 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; and
- protecting our rights in our intellectual property portfolio.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to continue to commercialize Translarna or to commence commercialization of EMFLAZA, either of which would have a material adverse effect on our business, results of operations and financial condition.

The marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD is limited to ambulatory patients aged five years and older located in the EEA, which significantly limits an already small treatable patient population, which reduces our commercial opportunity and is also subject to annual reassessment of the benefit-risk balance by the EMA as well as the specific obligation to conduct Study 041, and may be varied, suspended or withdrawn by the European Commission if we fail to satisfy those requirements.

We have obtained orphan drug designations from the EMA and from the FDA for Translarna for the treatment of nmDMD because the number of patients who could benefit from treatment with Translarna is small. The marketing label approved by the European Commission further limits the currently treatable patient population to ambulatory nmDMD patients aged five years and older who have been identified through genetic testing as having a nonsense mutation. Prevalence estimates for rare diseases are uncertain due to the uncertainties associated with the methodologies used to derive estimates, such as epidemiology assumptions. It can take many years of experience in rare disease market places before prevalence becomes well characterized. We are launching the first therapy specifically aimed at nmDMD patients. Our experience to date suggests that there may be up to 7,000 nmDMD patients globally and that approximately 35 to 40% of such patients satisfy the conditions for treatment under our current product label in the EEA, however, we expect that country specific epidemiology will continue to be refined and characterized over the coming years. Our estimates of both the number of people who have DMD caused by a nonsense mutation, as well as the subset of people with nmDMD who are ambulatory and at least five years old (and, therefore, satisfy the conditions for treatment under our current product label in the EEA), are based on our beliefs and estimates derived from a variety of sources and may prove to be incorrect. Prevalence estimates vary given some degree of variation in the incidence of live male births, the incidence of DMD, the incidence of nonsense mutations and other factors. Information concerning the eligible patient population is generally limited to certain geographies and may not employ definitive measures capable of establishing with precision the actual number of nmDMD patients in such geography. If the market opportunities for Translarna for the treatment of nmDMD are smaller than we believe they are, our business and anticipated revenues will be negatively impacted. Although we intend to seek to expand the approved product label of Translarna for the treatment of nmDMD in the future, the timing of, and our ability to generate, the necessary data or results required to obtain expanded regulatory approval is currently uncertain. Given the small number of patients who have nmDMD, and the smaller number of patients who meet the criteria for treatment under our current marketing authorization, our commercial opportunity is limited. It is critical to the commercial success of Translarna for nmDMD that we successfully identify and treat these patients.

Translarna is not approved, and is an investigational new drug, in the United States. In order to continue to generate revenue from Translarna, we must maintain our marketing authorization in the EEA for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. The marketing authorization in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment, as well as the specific obligation to complete and report the results of Study 041 to the EMA. We expect that as part of the annual EMA assessment, the EMA will consider the ongoing status of Study 041. The marketing authorization was last renewed in June 2017 and is effective, unless extended, through August 5, 2018. Enrolling Study 041 may further reduce the number of patients available for reimbursed treatment.

If the EMA determines in any annual renewal cycle that the balance of benefits and risks of using Translarna for the treatment of nmDMD has changed materially or that we have not or are unable to comply with any conditions that have been or may be placed on the marketing authorization, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require the imposition of other conditions or restrictions. As such, there is ongoing risk to our ability to maintain our marketing authorization in the EEA. If we are unable to renew our marketing authorization in the EEA during any annual renewal cycle, or if our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program, and in all territories, which would have a material adverse effect on our business, results of operations and financial condition. See "Risks Related to Regulatory Approval of our Products and our Product Candidates" below for further detail regarding conditional marketing authorizations in the EEA.

If clinical trials of Translarna or our product candidates fail to demonstrate safety and efficacy to the satisfaction of the EMA, the FDA or other regulators, or do not otherwise produce favorable results, we may experience delays in completing, or ultimately be unable to complete, the development and commercialization of Translarna or any other product candidate.

In connection with seeking marketing authorization from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of

preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing authorization of their products.

On March 2, 2017, we announced that the primary and secondary endpoints were not achieved in ACT CF and that, as a result, we plan to discontinue our current clinical development of Translarna for nmCF. We have withdrawn our type II variation submission with the EMA, which sought approval of Translarna for the treatment of nmCF in the EEA, and do not currently expect to pursue other marketing authorizations for this indication.

In addition, the primary efficacy endpoint in the ITT population did not achieve statistical significance in the Phase 2b (completed in 2009) or Phase 3 ACT DMD (completed in 2015) clinical trials of Translarna for the treatment of nmDMD. For a review of recent developments that have had, and may continue to have, a material adverse effect on our ability to commercialize Translarna for the treatment of nmDMD, please review the risk factor titled, "*ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations.*"

If the FDA, the EMA and other regulators do not agree with our interpretation of the results of the clinical data from our trials, including ACT DMD and, when and if completed, Study 041 and related analyses, or otherwise do not view the results of these trials as favorable; if we are required to conduct additional clinical trials or other testing of Translarna or any other product candidate that we develop beyond those that we contemplate; if we are unable to successfully complete our clinical trials or other testing; if the results of these trials or tests are not positive or are only modestly positive; or if there are safety concerns, we may, among other things:

- be unable to successfully maintain our marketing authorization in the EEA for Translarna for the treatment of nmDMD, which is subject to annual review and renewal following reassessment of the benefit-risk balance of the authorization by the EMA;
- be delayed in obtaining additional marketing authorizations, or not obtain additional marketing authorizations at all, for Translarna for the treatment of nmDMD;
- be delayed in obtaining marketing authorizations, or not obtain marketing authorizations at all, for Translarna for other indications, or for our other product candidates;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements or restrictions;
- have the product removed from markets after obtaining applicable marketing authorizations; or
- not be permitted to sell Translarna under some or any reimbursed EAP programs.

If we or our collaborators experience any of a number of possible unforeseen events in connection with clinical trials related to Translarna or our product candidates, including Study 041 and those under our collaboration with Roche and the SMA Foundation, maintenance of our existing marketing authorization for Translarna for the treatment of nmDMD in the EEA and any additional potential marketing authorization or commercialization of Translarna or our product candidates could be delayed or prevented.

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

- clinical trials of Translarna or our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product and product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- we may be unable to enroll a sufficient number of patients in our clinical trials to ensure adequate statistical power to detect any statistically significant treatment effects;

- we may enroll patients at clinical trial sites in countries that are inexperienced with clinical trials in general, or with the indication that is the subject of the trial;
- we may enroll patients at clinical trial sites in countries that have a different standard of care for patients in general, or with respect to the indication that is the subject of the trial;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, institutional review boards or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site or may require us to submit additional data, conduct additional studies or amend our investigational new drug application, or IND, or comparable application prior to commencing a clinical trial;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of Translarna or our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators, institutional review boards or independent ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of Translarna or our product candidates may be greater than we anticipate;
- the supply or quality of Translarna or our product candidates or other materials necessary to conduct clinical trials of Translarna or our product candidates may be insufficient or inadequate; or
- Translarna or our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the trials.

For example, the Phase 2 Moonfish study, which was evaluating the safety and efficacy of RG7800 under our SMA collaboration, was terminated in December 2016 following a suspension and clinical hold in the first half of 2015 to investigate an eye finding in a 39-week study in cynomolgus monkeys. The suspension and termination of Moonfish resulted in unanticipated delays in the advancement of the SMA program.

In addition, based on pre-clinical safety signals observed during the third quarter of 2015, we are no longer advancing PTC672 under our antibacterial program. Our product development costs will increase if we experience delays in testing or marketing authorizations. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize Translarna or our product candidates, allow our competitors to bring products to market before we do, or impair our ability to successfully commercialize Translarna or our product candidates, and so may harm our business, results of operations and financial condition.

Our conclusions regarding the activity and potential efficacy of Translarna in nmDMD are primarily based on retrospective, subgroup and meta-analyses of the results of our Phase 2b and ACT DMD clinical trials of Translarna for the treatment of nmDMD. Other than with respect to certain of our meta-analyses, results of our analyses are expressed as nominal p-values, which are generally considered less reliable indicators of efficacy than adjusted p-values. In addition, retrospective analyses are generally considered less reliable than pre-specified analyses.

After determining that we did not achieve the primary efficacy endpoint with the pre-specified level of statistical significance in our completed ACT DMD and Phase 2b clinical trials of Translarna for the treatment of nmDMD, we performed subgroup, retrospective, and meta-analyses. On the basis of our position that the totality of clinical data from these trials support the clinical benefit of Translarna for the treatment of nmDMD, we submitted these analyses to the FDA, as part of our NDA. In addition, after determining that the primary efficacy endpoint did not achieve statistical significance in ACT DMD or our Phase 2b clinical trial of Translarna for the treatment of nmDMD, we performed retrospective and subgroup analyses that we believe provide strong support for concluding that Translarna was active and showed clinically meaningful improvements over placebo in these trials.

Although we believe that these additional analyses of the results of these trials were warranted, a retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results.

Some of our favorable statistical data from these trials also are based on nominal p-values that reflect only one particular comparison when more than one comparison is possible. A p-value is called nominal if it is the result of one particular comparison prior to any pre-specified multiplicity adjustment, such as when two active treatments are compared to placebo or when two or more subgroups are analyzed. For example, while the p-values for change from baseline at week 48 in the 6-minute walk test, or 6MWT, which we also refer to as 6-minute walk distance, or 6MWD, and each secondary end point timed function test in the pre-specified subgroup of ACT DMD patients with a baseline 300-400 meter 6MWD had p-values of less than 0.05, due to the sequential testing method, these p-values are considered nominal.

Typically, a trial result is statistically significant if the chance of it occurring when the treatment is like placebo is less than one in 20, resulting in a p-value of less than 0.05. Nominal p-values cannot be compared to the typical significance level (p-value less than 0.05) to determine statistical significance without being adjusted for the testing of multiple dose groups, end points or analyses of subgroups.

Because of these limitations, regulatory authorities typically give greater weight to results from pre-specified analyses and adjusted p-values and less weight to results from post-hoc, retrospective analyses and nominal p-values.

On February 22, 2016, we received a Refuse to File letter from the FDA stating that, in the view of the FDA, both the Phase 2b and Phase 3 ACT DMD trials were negative and do not provide substantial evidence of effectiveness and that our NDA does not contain adequate information regarding the abuse potential of Translarna. Additionally, the FDA stated that we had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. Our reliance on nominal p-values for some of our statistical data and our use of retrospective analyses had a negative impact on the FDA's view of our interpretation of the results of our Phase 2b trial, ACT DMD and the totality of data from our clinical trials.

Although we filed our NDA over protest with the FDA, there is substantial risk that, notwithstanding any dialogue we have had or any further dialogue we may be able to initiate with the agency, pursuant to the file over protest process or otherwise, the FDA will continue to disagree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials. Even if we are successful in resolving some or all of the matters raised by the FDA in the Refuse to File letter, there is significant risk that we will be unable to obtain FDA approval of Translarna for nmDMD, on a timely basis or at all, and we may be required to perform additional clinical and non-clinical trials or complete additional analyses at significant cost. Even if we are able to enroll and fund any such additional trials or complete such analyses, there is substantial risk that the results would not ultimately support the approval of the NDA filed over protest or a new NDA submission in the United States for Translarna for nmDMD. In addition, any such requirement for additional trials would most likely result in our inability to sell Translarna in the United States for a significant period of time, which would have a material adverse effect on our ability to generate revenue from the sales of Translarna for the treatment of nmDMD.

Our reliance on nominal p-values for some of our statistical data and our use of retrospective analyses has also had a negative impact on the EMA's evaluation of our last application for continued marketing authorization for Translarna for the treatment of nmDMD, including delays in timing of the CHMP's opinion with respect to the annual renewal of our marketing authorization, and could negatively impact regulatory determinations by regulators in other territories.

An unfavorable view of our data and analyses by the FDA and EMA for Translarna has and could continue to negatively impact our ability to obtain or maintain authorizations to market Translarna for the treatment of nmDMD. An inability to obtain new marketing authorizations or maintain our current marketing authorization in the EEA would have a material adverse effect on our revenue from Translarna and would materially harm our business, financial results and results of operations.

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

There are no marketed therapies approved to treat the underlying cause of nmDMD. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat nmDMD and other diseases that we are studying or have studied, including, nmCF, nmMPS I, nonsense mutation aniridia, and nonsense mutation Dravet syndrome/CDKL5. As a result, the design and conduct of clinical trials for these diseases, particularly for drugs to address the underlying nonsense mutations causing these diseases in some subsets of patients, is subject to increased risk.

For example, on March 2, 2017, we announced that the primary and secondary endpoints were not achieved in ACT CF, our Phase 3 clinical for Translarna in nmCF and that, as a result, we plan to discontinue our current clinical development of Translarna for nmCF.

Prior to the Phase 2b clinical trial of Translarna for nmDMD, there was no precedent of an established trial design to evaluate the efficacy of Translarna in nmDMD over a 48 week duration. In addition, clinical understanding of the methodologies used to analyze the resulting data were also limited. The study design and enrollment criteria for ACT DMD were based on available natural history data of the disease, including third-party data and results from our Phase 2b clinical trial. An evolving understanding in the DMD community has led to a greater appreciation of the optimal window for the 6MWT in assessing physical function. We believe that this factor may have led to the primary efficacy endpoint in the intent to treat population not achieving statistical significance in ACT DMD.

We are faced with similar challenges in connection with the design of our studies of Translarna in nonsense mutation aniridia, and nonsense mutation Dravet syndrome/CDKL5, and such similar challenges existed in our study of nmMPS I, which study we recently decided will be stopped, because there is also limited historical clinical trial experience for the development of drugs to treat the underlying cause of these disorders.

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

We may not be able to initiate or continue clinical trials for our product candidates, including Study 041 or our Phase 2 studies of Translarna in nonsense mutation aniridia or nonsense mutation Dravet syndrome/CDKL5, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. The studies under our SMA collaboration face similar risks.

Each of the indications we are currently pursuing for Translarna and our product candidates are characterized by relatively small patient populations, which could result in slow enrollment of clinical trial participants. The feasibility of patient enrollment was a critical factor discussed with the EMA in connection with the specific obligation to conduct Study 041, in particular due to factors that increase the challenges of enrollment, such as the small nmDMD patient population, the patient eligibility criteria for the mITT for Study 041, and the fact that Translarna is available to patients in the EEA and other limited territories pursuant to commercial and EAP programs.

In addition, our competitors have ongoing clinical trials for product candidates that could be competitive with our product candidates. As a result, potential clinical trial sites may elect to dedicate their limited resources to participation in our competitors' clinical trials and not ours, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is affected by other factors including:

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

Enrollment delays in our clinical trials may result in increased development costs for our product candidates. Our inability to enroll a sufficient number of patients in Study 041 or our Phase 2 studies of Translarna in nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 or any of our, or our collaboration partners', other clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. As the conduct of Study 041 is a specific obligation to our marketing authorization in the EEA for Translarna for the treatment of nmDMD, any such delay or inability to enroll sufficient patients could have a material adverse effect on our ability to maintain our authorization in the EEA and, failure to maintain such authorization would have a material adverse effect on our business, results of operations and financial performance.

For example, we amended the study design for our proof-of-concept study for Translarna for the treatment of nmMPS I to include patients currently on enzyme replacement therapy, which contributed to delays in site initiation and patient accrual. Despite the protocol amendment, we continued to encounter difficulties identifying qualified patients for this study and recently decided that we will be stopping the study due to the lack of patients.

If serious adverse or inappropriate side effects are identified during the development of Translarna or any other product candidate or for any product for which we have or may obtain marketing approval, including Translarna and EMFLAZA, we may need to abandon or limit our development and/or marketing of that product or product candidate.

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

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

In addition, in Study 009, our first Phase 3 clinical trial of Translarna for the treatment of nmCF, five adverse events in the Translarna arm of the trial that involved the renal system led to discontinuation. As compared to the placebo group, the Translarna treatment arm also had a higher incidence of adverse events of creatinine elevations, which can be an indication of impaired kidney function. In the Translarna treatment arm, more severe clinically meaningful creatinine elevations were reported in conjunction with cystic fibrosis pulmonary exacerbations. These creatinine elevations were associated with concomitant treatment with antibiotics associated with impaired kidney functions, such as aminoglycosides or vancomycin. This led to the subsequent prohibition of concomitant use of Translarna and these antibiotics, which was successful in addressing this issue in the clinical trial.

In addition, we are obligated to perform certain FDA post-marketing requirements in connection with our marketing authorization of EMFLAZA in the United States, including pre-clinical and clinical safety studies. If we or others identify previously unknown side effects, whether pursuant to these post-marketing requirements, or otherwise, and in particular if such side-effects are severe, or if known side effects are more frequent or severe than in the past then our marketing authorization for EMFLAZA may be restricted or withdrawn, changes may be required to the product's label, sales may be adversely impacted, we may be required to undertake additional studies or trials, and government investigations or litigation, including product liability claims, may be brought against us. This is the first time EMFLAZA will be used on a common use basis, and unknown safety findings may arise through common use, which may lead EMFLAZA to be restricted or withdrawn, changes may be required to the product's label, sales may be adversely impacted, we may be required to undertake additional studies or trials, and government investigations or litigation, including product liability claims, may be brought against us. Additionally, if the safety warnings in our product labels are not followed, adverse medical situations in patients may arise, resulting in negative publicity and potential lawsuits, even if our products worked as we described. Any of these occurrences would limit or prevent us from commercializing EMFLAZA, which would have a material adverse effect on our business, financial results and operations.

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

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

For example, on March 2, 2017, we announced that the primary and secondary endpoints were not achieved in ACT CF and that, as a result, we plan to discontinue our current clinical development of Translarna for nmCF. We have withdrawn our type II variation submission with the EMA, which sought approval of Translarna for the treatment of nmCF in the EEA.

In addition, although we have received marketing authorization by the European Commission for Translarna for the treatment of nmDMD, such marketing authorization is subject to the specific obligation to conduct and submit the results of Study 041 to the EMA and is also subject to annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA. In 2016, the FDA refused to file our NDA for Translarna for the treatment of

nmDMD, noting that both the Phase 2b and Phase 3 ACT DMD trials of Translarna for the treatment of nmDMD were negative and do not provide substantial evidence of effectiveness.

We may not be successful in renewing our marketing authorization for Translarna for the treatment of nmDMD in the EEA or in obtaining full regulatory approval for Translarna for the treatment of nmDMD or any indication or for any other potentially commercially viable drug that treats an approved indication by targeting a particular post-transcriptional control process. Furthermore, we may not receive regulatory approval for product candidates that target different post-transcriptional control processes. If we fail to develop and commercialize viable drugs, we will not achieve commercial success.

Translarna for the treatment of nmDMD, EMFLAZA for the treatment of DMD, or any other product candidate that receives marketing authorization, if any, may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Although Translarna is currently authorized by the EMA for marketing for the treatment of nmDMD such marketing authorization is subject to the specific obligation to conduct and submit the results of Study 041 to the EMA and is also subject to annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA. Even if our marketing authorization in the EEA for Translarna for the treatment of nmDMD is maintained, or we are successful in obtaining marketing authorization for Translarna for other indications or territories or marketing authorization for any of our other product candidates, such product may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. In addition, EMFLAZA for the treatment of DMD in the United States may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Third-party payers may require prior authorizations or failure on another type of treatment before covering a particular drug, particularly with respect to higher-priced drugs. Decreases in third-party reimbursement for a product or a decision by a third-party payor to not cover a product could reduce physician usage of the product, including EMFLAZA or Translarna. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or any profits from operations.

The degree of market acceptance of our products or product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects;
- the ability to offer our products or product candidates for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement;
- adverse publicity about our products or product candidates or favorable publicity about competitive products or product candidates; and
- any restrictions on concomitant use of other medications.

In addition, because we are developing Translarna for the treatment of different indications, negative results in a clinical trial evaluating the efficacy of Translarna for one indication, such as our recent ACT CF trial results, could have a negative impact on the perception of the efficacy of Translarna in a different indication, which could have an adverse effect on our commercialization efforts and financial results.

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

If we are unable to establish sales, marketing and distribution capabilities or enter into agreements with third parties to market, sell and distribute our products or product candidates, we may not be successful in our continuing efforts to commercialize Translarna or EMFLAZA or commercializing any other product candidate if and when they are approved.

Our past experience in the sale, marketing and distribution of pharmaceutical products is limited to our activities under the marketing authorization in the EEA for Translarna for the treatment of nmDMD, which is subject to annual review and renewal following reassessment of the benefit-risk balance of the authorization by the EMA and satisfaction of the specific obligation to conduct Study 041.

Our commercial launch of EMFLAZA for DMD represents our first product launch in the United States. We may be unable to successfully execute our commercial strategy for EMFLAZA for the treatment of DMD in the United States or Translarna for the treatment of nmDMD in other territories, including, if approved, in the United States, or for other indications or product candidates that may receive marketing authorization, if any.

Our ongoing commercial strategy for our products involves the development of a commercial infrastructure that spans multiple jurisdictions and is heavily dependent upon our ability to continue to build an infrastructure that is capable of implementing our global commercial strategy. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to develop our commercial organizations in all intended territories, including in the United States, in a timely manner or at all. Doing so will require a high degree of coordination and compliance with laws and regulations in numerous territories, including in the United States, each state, including restrictions on advertising practices, enforcement of intellectual property rights, restrictions on pricing or discounts, and unexpected changes in regulatory requirements and tariffs. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize EMFLAZA in the United States and Translarna in the EEA and other jurisdictions in which it is or may be available will be adversely affected. If we are unable to establish and maintain adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue consistent with our expectations and may not become profitable.

We intend to continue to promote Translarna for the treatment of nmDMD in permitted territories using both internal and external resources. We also intend to utilize both internal and external resources in connection with our ongoing commercial launch of EMFLAZA in the United States.

There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training an internal commercial team is expensive and time consuming and could delay commercialization efforts. If a commercial launch for any product or product candidate for which we recruit a commercial team and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition such personnel.

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

If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our products or product candidates.

Factors that may materially affect our efforts to commercialize our products include:

- our ability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our ability to attract, retain and assimilate key personnel in connection with our acquisition, integration and commercialization of EMFLAZA;
- our ability to replace services being performed pursuant to a transition services agreement with Marathon before the termination of such agreement;
- our ability to implement third-party marketing and distribution relationships on favorable terms, or at all, in territories where we do not pursue direct commercialization;
- the ability of our commercial team to obtain access to or persuade adequate numbers of physicians to prescribe Translarna, EMFLAZA or any future products;
- the lack of complementary products to be offered by our commercial team, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercial organization.

Any of these factors, individually or as a group, if not resolved in a favorable manner may have a material adverse effect on our business and results of operations. Similar risks apply in those territories where Translarna is available on a reimbursed basis under an EAP program.

All of our sales of Translarna for the treatment of nmDMD currently occur in territories outside of the United States, which subjects us to additional business risks that could adversely affect our revenue and results of operations.

All of our revenue from sales of Translarna to date has been generated from countries other than the United States. We have operations in multiple European countries and other territories, including Latin America. We expect that we will continue to expand our international operations in the future, including in emerging growth markets, pending successful completion of the applicable regulatory processes. International operations inherently subject us to a number of risks and uncertainties, including:

- political, regulatory, compliance and economic developments that could restrict our ability to manufacture, market and sell our products;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates;
- difficulty in staffing and managing international operations;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- trade protection measures, including import or export licensing requirements and tariffs;
- our ability to develop relationships with qualified local distributors and trading companies;
- political and economic instability in particular foreign economies and markets, in particular in emerging markets;
- diminished protection of intellectual property in some countries outside of the United States;
- differing labor regulations and business practices; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act, UK Bribery Act or similar local regulation.

For example, we face risks arising out of the potential uncertainty caused by the vote in the United Kingdom in favor of exiting the European Union, commonly referred to as Brexit. Brexit could adversely affect European or worldwide political, regulatory, economic or market conditions and could contribute to instability in global political institutions, regulatory agencies and financial markets. Currency exchange rates in the pound sterling and the euro with respect to each other and the U.S. dollar have already been adversely affected by Brexit and, in the event that such foreign exchange volatility were to continue, it could cause volatility in our quarterly financial results. In addition, if the United Kingdom were to significantly alter its regulations affecting the pharmaceutical industry, we could face significant new regulatory costs and challenges.

In addition, some of the countries in which Translarna for the treatment of nmDMD is available for sale are in emerging markets. Some countries within emerging markets may be especially vulnerable to periods of global or regional financial instability or may have very limited resources to spend on health care, including Brazil. We also may be required to increase our reliance on third-party agents within less developed markets. In addition, many emerging market countries have currencies that fluctuate substantially and if such currencies devalue and we cannot offset the devaluations, our financial performance within such countries could be adversely affected.

In addition, in some countries, including those in Latin America, orders for named patient sales may be for multiple months of therapy, which can lead to an unevenness in orders which could result in significant fluctuations in quarterly net product sales. Other factors may also contribute to fluctuations in quarterly net product sales including Translarna's availability in any particular territory, government actions, economic pressures, political unrest and other factors. Net product sales are impacted by factors, such as the timing of decisions by regulatory authorities, in particular the FDA and the EMA with respect to our ability to market or sell Translarna for the treatment of nmDMD, and our ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which we have or may obtain regulatory approval, including the United States, EEA and other territories.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations.

As we continue to expand our existing international operations, we may encounter new risks.

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

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

There is currently no marketed therapy, other than Translarna in the EEA, which has received approval for the treatment of the underlying cause of nmDMD. Sarepta Therapeutics recently received approval in the United States for a treatment addressing the underlying cause of disease for different mutations in the DMD gene. Other biopharmaceutical companies are developing treatments for the underlying cause of disease for different mutations in the DMD gene (Sarepta, Daiichi Sankyo, and Nippon Shinyaku).

Aldurazyme, which is manufactured by BioMarin Pharmaceutical Inc. and sold by Genzyme Corporation, is an enzyme replacement therapy for the treatment of mucopolysaccharidosis I. Furthermore, Diacomit is marketed in the European Union by Laboratoires Biocodex for the treatment of Dravet syndrome. Other companies are also pursuing product candidates for the treatment of Dravet syndrome, including GW Pharmaceuticals, Zogenix, and Insys Therapeutics. Aniridia therapeutic interventions, such as artificial iris implantation, are being developed by HumanOptics AG. Our SMA collaboration with Roche and the SMA Foundation also faces competition. For example, in December 2016, the FDA approved nusinersen, a drug developed by Ionis Pharmaceuticals, Inc. and marketed by Biogen, to treat SMA. AveXis, Inc. is also evaluating a gene therapy product candidate for the treatment of SMA. Other companies are also pursuing product candidates for the treatment of SMA, including Trophos (also in collaboration with Roche), Kowa, Novartis Pharmaceuticals Corporation, and Cytokinetics.

Although EMFLAZA is currently the only corticosteroid approved for the treatment of DMD in the United States, prednisone another corticosteroid drug, is approved for other indications in the United States and may be prescribed for DMD patients and we expect that we will compete with this treatment following our commercial launch of EMFLAZA.

Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are marketing or developing or that would render our products or product candidates obsolete or non-competitive. Our competitors may also obtain marketing authorization for their products more rapidly than we may obtain approval for our products and product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market.

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

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

Even if we are able to commercialize Translarna for the treatment of nmDMD on a broad scale, commercialize EMFLAZA for the treatment of DMD in the United States or commercialize Translarna for other indications or any other product candidate that we develop, Translarna, EMFLAZA and any other product or product candidate may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

We may not obtain adequate coverage or reimbursement for our products, including EMFLAZA and Translarna, or we may be required to sell our products at an unsatisfactory price. In addition, obtaining pricing, coverage and reimbursement approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we do not receive these approvals on a timely basis.

The regulations and practices that govern marketing authorizations, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries, including almost all of the member states of the EEA, require approval of the sale (list) price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, including the European

market, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing authorization for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more products, including EMFLAZA and Translarna or other product candidates, even following marketing authorization.

Our ability to successfully commercialize Translarna, EMFLAZA or any other product candidate that receives marketing authorization will depend in large part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, managed health care organizations and other third-party payors and organizations. Government authorities and other third-party payors, such as private health insurers and managed health care organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the EU and U.S. healthcare industries and elsewhere is cost containment. Government authorities, including the United States government and state legislatures, and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Prices at which our products are reimbursed can be subject to challenge, reduction or denial by the government and other payers. Increasingly, third-party payors are requiring that drug companies provide them with discounts off the products' sale (list) prices and are challenging the prices manufacturers charge for medical products. We cannot be sure that coverage will be available for Translarna, EMFLAZA or any other product that we may commercialize and, if coverage is available, the level of reimbursement is also uncertain.

Reimbursement may impact the demand for, or the price of, any product or product candidate for which we obtain marketing authorization. Obtaining reimbursement for EMFLAZA and for Translarna has been and is expected to continue to be, particularly difficult due to price considerations typically associated with drugs that are developed to treat conditions that affect a small population of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug, such as prior authorization and the requirement to try other therapies first. Decreases in third-party reimbursement for a product or a decision by a third-party payor to not cover a product could reduce physician usage of the product, including EMFLAZA or Translarna. If reimbursement is not available or is available only on a limited basis, we may not be able to successfully commercialize any product or product candidate for which we have obtained or may obtain marketing authorization, including EMFLAZA or Translarna.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Further, coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws, enforcement policies or administrative determinations that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States.

In the United States, third-party payors include federal health care programs, such as Medicare, Medicaid, TRICARE, and Veterans Health Administration programs; managed care providers, private health insurers and other organizations. Several of the U.S. federal health care programs require that drug manufacturers extend discounts or pay rebates to certain programs in order for their products to be covered and reimbursed. For example, drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule, or FSS. FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the United States Public Health Service, or PHS, 340B drug pricing program. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the "federal ceiling price") and may be subject to an additional discount if pricing increases more than the rate of inflation.

In addition, while EMFLAZA is eligible to be reimbursed by Medicaid, the Medicaid Drug Rebate Program requires pharmaceutical manufacturers of covered outpatient drugs to enter into and have in effect a national rebate agreement with the federal government as a condition for coverage of the manufacturer's covered outpatient drug(s) by state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases

more than inflation. State Medicaid programs and Medicaid managed care plans can seek additional "supplemental" rebates from manufacturers in connection with favorable positioning on formularies.

Similarly, in order for a covered outpatient drug to receive federal reimbursement under the Medicare Part B and Medicaid programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts on the covered outpatient drug to entities that are enrolled and participating in the 340B drug pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

EMFLAZA is also eligible for reimbursement under the Medicare Part D program established by The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities, which will provide coverage of outpatient prescription drugs. Part D prescription drug formularies are required to include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain.

In addition, U.S. private health insurers often rely upon Medicare coverage policies and payment limitations in setting their own coverage and reimbursement policies. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors. Payment by private payors is also subject to payor-determined coverage and reimbursement policies that vary considerably and are subject to change without notice. We expect that coverage and reimbursement of EMFLAZA in the United States will vary from commercial payor to commercial payor. Many commercial payors, such as managed care plans, manage access to FDA approved product coverage partly to control costs to their plans, and may use drug formularies and medical policies to limit their exposure. Exclusion from policies can directly reduce product usage in the payor's patient population and may negatively impact utilization in other payor plans, as well.

There has been recent negative publicity and increasing legislative and public scrutiny around pharmaceutical drug pricing in the U.S., in particular with respect to orphan drugs and specifically with respect to EMFLAZA. Moreover, U.S. government authorities and third-party payors are increasingly attempting to limit or regulate drug prices and reimbursement, often with particular focus on orphan drugs. These dynamics may give rise to heightened attention and potential negative reactions to pricing decisions for EMFLAZA and products for which we may receive regulatory approval in the future, possibly limiting our ability to generate revenue and attain profitability.

Moreover, we expect that the new Presidential Administration and U.S. Congress may seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the 2010 U.S. healthcare reform legislation (the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, known collectively as the Affordable Care Act), which could have an impact on coverage and reimbursement for healthcare items and services covered by the federal and state healthcare programs as well as plans in the private health insurance market. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices with respect to Translarna for the treatment of nmDMD and other product candidates that might receive marketing authorization in the future. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for our product or any of our product candidates that may receive marketing authorization, or a reduction in coverage for payment rates for our product or any such product candidates, could have a material adverse effect on our business, results of operations and financial condition. In addition, in the European Union, for medicines authorized by the centralized authorization procedure, an authorized trader, such as a wholesaler, can purchase a medicine in one EU member state and import the product into another EU member state. This process is called "parallel distribution". As a result, a purchaser in one EU member state may seek to import Translarna from another EU member state where Translarna is sold at a lower price. This could have a negative impact on our business, financial condition, results of operations and growth.

Similarly, sales of EMFLAZA in the United States could also be reduced if deflazacort is imported into the United States from lower-priced markets, whether legally or illegally. For example, in the United States, prices for pharmaceuticals are generally higher than in the bordering nations of Mexico and Canada. There have been proposals to legalize the import of pharmaceuticals from outside the United States. If such legislation were enacted, our revenues from EMFLAZA could be reduced, and our business, results of operations and financial condition could be materially adversely affected.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit clinical trials or commercialization of any current or future products.

We face an inherent risk of product liability exposure related to the commercialization of Translarna, EMFLAZA and any other product that we may market or commercialize, and in connection with the human clinical trials testing of our products or

product candidates. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for our products or any product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- the inability to continue current clinical trials or begin planned clinical trials;
- withdrawal or reduced enrollment of clinical trial participants;
- significant costs to defend the related claims/litigation;
- increased insurance costs, or an inability to maintain appropriate insurance coverage;
- substantial monetary awards to trial participants or patients or their families;
- loss of revenue; and
- the inability to commercialize or to continue commercializing any products or product candidates.

We have product liability insurance that covers our commercial sales, sales pursuant to reimbursed EAP programs and clinical trials up to a $25.0 million annual aggregate limit, and subject to a per claim deductible, and which has recently been extended to cover corresponding risks for EMFLAZA. The amount of insurance we currently hold may not be adequate to cover all liabilities and defense costs that we may incur. We may need to further increase our insurance coverage as we commercialize Translarna and EMFLAZA, or as and when we begin commercializing any other product candidate that receives marketing authorization. The cost of insurance coverage is highly variable, based on a wide range of factors. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability or defense costs that may arise.

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

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

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We also maintain liability insurance for some of these risks, but our liability policy excludes pollution and has an aggregate coverage limit of $11.0 million.

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

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

Because we have limited financial and managerial resources, we focus on products, research programs and product candidates for specific indications. As a result, we may forgo or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential.

For example, in connection with our acquisition of EMFLAZA, we paid upfront consideration comprised of $75 million in cash, funded through cash on hand, and 6,683,598 shares of our common stock. We are also required to make contingent payments to Marathon based on annual net sales of EMFLAZA beginning in 2018, up to a specified aggregate maximum amount for such payments, and a single $50 million sales-based milestone, in each case subject to the terms and conditions of

the Asset Purchase Agreement. We may never realize the anticipated benefits of the acquisition of EMFLAZA and by investing our limited resources in this product, we may be required to forgo or delay other opportunities.

In addition, we initiated separate Phase 2 clinical trials of Translarna for the treatment of hemophilia in 2009 and the metabolic disorder methylmalomic acidemia in 2010, but then suspended these clinical trials to focus on the development of Translarna for nmDMD and nmCF when we found variability in the assays used in these trials and preliminary data from these trials did not indicate definitive evidence of activity. In March 2017, we discontinued our current clinical development of Translarna for nmCF based on the negative outcome of a Phase 3 clinical trial. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

We have based our research and development efforts on small-molecule drugs that target post-transcriptional control processes. Notwithstanding our large investment to date and anticipated future expenditures in proprietary technologies, including our GEMS, nonsense mutation and alternative splicing technologies, which we use in the discovery of these molecules, to date we have only been granted marketing authorization in the EEA to treat nmDMD under a restricted label that is subject to the specific obligation to conduct Study 041 as well as annual renewal and reassessment requirements. We may not be able to successfully renew or satisfy the ongoing requirements of our current marketing authorization for nmDMD in the EEA and we may never successfully develop any other marketable drugs or indications using our scientific approach. As a result of pursuing the development of product candidates using our proprietary technologies, we may fail to develop product candidates or address indications based on other scientific approaches that may offer greater commercial potential or for which there is a greater likelihood of success. Research programs to identify new product candidates require substantial technical, financial and human resources. These research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development.

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

Risks Related to Regulatory Approval of our Products and our Product Candidates

Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon our ability to satisfy the specific obligation to conduct and report the results of Study 041 by September 2021, and, as such, there is ongoing risk that we may be unable to maintain such authorization. If we are unable to obtain renewal of such marketing authorization in any future renewal cycle, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, financial performance and results of operations.

Conditional marketing authorizations based on incomplete clinical data, including our marketing authorization for Translarna for the treatment of nmDMD, may be granted in the EEA for a limited number of listed medicinal products for human use, including products designated as orphan medicinal products under EU law, if (1) the EMA determines that the benefit-risk balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) unmet medical needs will be fulfilled and (4) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations or conditions, including with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data, may be specified in the conditional marketing authorization. Marketing authorizations subject to conditions are only valid for one year, and must be renewed annually by the European Commission after an assessment by the EMA of the ongoing positive benefit-risk balance in favor of continued authorization and the need for additional or modified conditions.

We received initial marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older from the European Commission in August 2014 as a "conditional marketing authorization." The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon our satisfaction of the specific obligation to conduct and submit the results of Study 041 by September 2021 to the EEA. We expect that as part of the annual EMA assessment, the EMA will consider the ongoing status of Study 041. We are also required to implement measures, including pharmacovigilance plans, which are detailed in the risk management plan.

Our marketing authorization was previously conditioned upon our submission to the EMA of the final efficacy and safety report from ACT DMD during 2015. Although we have fulfilled the condition to submit the ACT DMD report to the EMA, that trial

did not meet the primary efficacy endpoint of change from baseline at week 48 in distance walked in the 6-minute walk test. The EMA and European Commission did not approve our request for full marketing authorization of Translarna for the treatment of nmDMD and, instead, approved the renewal of our conditional marketing authorization with the specific obligation to confirm the efficacy and safety of Translarna for the treatment of nmDMD in ambulatory patients aged 5 years or older via Study 041.

Enrolling, conducting and reporting a clinical trial is a time-consuming, expensive and uncertain process that takes years to complete, and we expect that we will incur material costs related to the implementation and conduct of Study 041. We expect that conducting a placebo-controlled trial in nmDMD of this size will be challenging and it is probable that we will enroll patients in territories where Translarna has already become available on a reimbursed basis. We may enroll patients in countries with a different standard of care for nmDMD patients or at clinical trial sites that are inexperienced with clinical trials in general, or specifically with nmDMD trials. In addition, we may experience unknown complications with Study 041 and may not achieve the pre-specified endpoint with statistical significance, which would have a materially adverse effect on our ability to maintain our marketing authorization in the EEA.

If we fail to satisfy our obligations under the marketing authorization, or if it is determined in any annual renewal cycle that the balance of benefits and risks of using Translarna has changed materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna. The EMA may also impose other new conditions to our marketing authorization (in addition to Study 041), and may make other recommendations, including new label restrictions. In the event that we do secure annual renewal of the marketing authorization for any given annual renewal cycle, the EMA could nevertheless later determine that we have not complied, or are unable to comply, with any conditions that have been or may be placed on the marketing authorization, including those related to Study 041, which could result in the withdrawal of our marketing authorization or other outcome that would have a materially adverse effect on our business, results of operations and financial condition.

If our marketing authorization in the EEA is not renewed, or our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, results of operations and financial condition.

There is substantial risk that the FDA will continue to disagree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD and we will be unable to advance Translarna for the treatment of nmDMD in the United States in a timely manner, or at all, whether pursuant to the file over protest process or otherwise, and by determining to file our NDA over protest, we have postponed other available strategic pathways which may have proven to be more effective. If there are delays in obtaining regulatory approval in the United States, we will not be able to commercialize Translarna for nmDMD in that territory and our ability to generate revenue will be materially impaired. In the event that the FDA requires us to conduct a new clinical trial in nmDMD which, if successful, may enable FDA review of an NDA submission by us, we would expect to incur significant costs, which may have a material adverse effect on our business and results of operations.

In December 2015, we completed our rolling new drug application, or NDA, for Translarna for the treatment of nmDMD with the FDA. In February 2016, we received a Refuse to File letter from the FDA regarding this NDA. The FDA stated in the Refuse to File letter that our NDA was not sufficiently complete to permit a substantive review. Specifically, we were notified in the letter that, in the view of the FDA, both of our Phase 2b and Phase 3 ACT DMD trials of Translarna for the treatment of nmDMD were negative and do not provide substantial evidence of effectiveness. Additionally, the FDA stated that we had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. During the third quarter of 2016, we filed an appeal of the FDA's decision refuse to file the NDA, which was denied in the fourth quarter of 2016.

Rather than continue our appeal under the formal dispute resolution process, we recently filed the NDA over protest with the FDA and the agency granted a standard review for the NDA and has set a PDUFA target review date of October 24, 2017, which is not binding on the agency, and has tentatively scheduled an advisory committee meeting on September 28, 2017 to facilitate its review. There can be no assurance that the FDA will complete its review of our NDA by the PDUFA goal date.

Filing over protest is a procedural path permitted by FDA regulations that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission. When an application is filed over protest, the FDA is required to review the application as filed. In the case of our Translarna NDA, the FDA permitted us to update certain aspects of the filing relating to evidence of efficacy in connection with the filing over protest, but these updates may not prove persuasive to FDA in considering whether to approve our NDA. Generally, the FDA does not favor the file over protest procedure and the agency's policies explain that an application filed over protest does not receive a timeline for review and is designated as a standard review.

There is substantial risk that, notwithstanding any dialogue we have had or any further dialogue we may be able to initiate with the agency, pursuant to the file over protest process or otherwise, we will continue to be unable to resolve the matters raised by the FDA in its Refuse to File letter in a timely manner, or at all. Even if we are successful in resolving some or all of those matters, there is significant risk that the FDA will continue to disagree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials and we will be unable to obtain FDA approval of Translarna for nmDMD, on a timely basis or at all. We may be required to perform additional clinical and non-clinical trials or analyses at significant cost. Even if we are able to enroll and fund any such additional trials or analyses, there is substantial risk that the results would not ultimately support the approval of the NDA filed over protest or a new NDA submission with the FDA for Translarna for the treatment of nmDMD in the United States. In addition, any such requirement for additional trials would most likely result in our inability to sell Translarna in the United States for a significant period of time, if ever, which would have a material adverse effect on our ability to generate revenue from the sales of Translarna for the treatment of nmDMD.

For example, we filed a formal dispute resolution request with the FDA in connection with the agency's refuse to file our NDA submitted for approval of Translarna for the treatment of nmDMD that was based on our Phase 2b data. In January 2012, the FDA reaffirmed the appropriateness of its earlier decision to refuse to file the 2011 NDA. In February 2012, we discussed the design of a proposed Phase 3 clinical trial with the FDA. In that meeting, although the FDA indicated that the adequacy of data for filing and approval of an NDA would remain review issues, the FDA had no objections to key elements of our proposed trial design. We ultimately submitted the safety and efficacy data of that Phase 3 trial, ACT DMD, as part of the NDA that is the subject of the current file over protest procedure. In its 2016 Refuse to File letter, the FDA referenced its prior refusal to file relative to the Phase 2b data and our discussions with the FDA, reiterating the views previously disclosed.

Furthermore, we expect that our efforts to advance our regulatory strategy in the United States will be time-consuming and may be expensive. In addition, by determining to discontinue our strategy of appealing the FDA's refusal to file our NDA and pursue the file over protest strategy, we have postponed other strategic pathways, such as escalating our appeal to the next supervisory level of the FDA, commencing direct litigation, or discussing the design of a new clinical trial with the FDA. Such alternative strategies may be more effective than the file over protest procedure in achieving our ultimate goal of approval for Translarna for the treatment of nmDMD in the United States. We will not be able to commercialize Translarna for nmDMD in the United States until we have obtained regulatory approval from the FDA. Delays in obtaining such approval will materially impair our ability to generate revenue from Translarna for the treatment of nmDMD.

For additional information concerning recent developments that have had, and may continue to have, a material adverse effect on our ability to advance our regulatory strategy for Translarna for the treatment of nmDMD, please review the risk factor under "Risks Related to the Development and Commercialization of our Products and our Product Candidates" titled, "*ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations.*"

If we are not able to comply with local regulations for our products or product candidates, we will not be able to obtain or maintain product approvals and commercialize our product or product candidates, and our ability to generate revenue will be materially impaired.

Translarna, EMFLAZA and our product candidates, and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and EMA and by comparable authorities in other countries. Failure to obtain or renew marketing authorization for Translarna or any product candidate, or maintain our marketing authorization for EMFLAZA in the United States will prevent us from commercializing such product or product candidate.

As noted in the foregoing risk factors, we may not receive necessary approvals from the FDA, the EMA, or other regulators to further commercialize Translarna for nmDMD or to commercialize Translarna for any other indication or to commercialize any product candidate in any market. For example, in March 2017, we discontinued our current clinical development of Translarna for nmCF based on the outcome of our Phase 3 clinical trial, ACT CF, and have withdrawn our type II variation submission with the EMA, which we had commenced in the third quarter of 2015 to seek approval of Translarna for the treatment of nmCF in the EEA. We do not currently expect to pursue other marketing authorizations for this indication.

We have not proven our ability to successfully obtain marketing authorizations to sell our product or product candidates, other than with respect to the marketing authorization granted by the European Commission in August 2014 for Translarna for the treatment of nmDMD, which is subject to annual review and renewal following reassessment of the benefit-risk balance of the authorization by the EMA and satisfaction of any conditions that may be imposed by the EMA, including the specific obligation to conduct and report the results of Study 041 and our marketing authorizations in Israel and South Korea (which are largely contingent upon continued EMA approval). We have begun seeking and intend to continue to seek marketing

authorization for Translarna for the treatment of nmDMD in territories outside of the EEA. There is substantial risk that regulators in other territories will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials, which would have a material adverse effect on our ability to generate revenue, or may prevent us from generating any revenue, from the sales of Translarna for the treatment of nmDMD in those territories.

We have only limited experience in filing and supporting the applications necessary to obtain marketing authorizations for product candidates and expect to continue to rely on third-party contract research organizations to assist us in this process. Securing marketing authorization requires the timely preparation and submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. In response to changes in the regulatory environment or requests from regulators, we may elect, or be obliged, to postpone a regulatory submission to include additional analyses, including those intended to strengthen our submission or facilitate regulator review, which could cause delays in getting our products to market and substantially increase our costs. Securing marketing authorization also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that Translarna or any of our other product candidates are not effective or only moderately effective, or have undesirable or unintended side effects, toxicities, safety profiles or other characteristics that preclude us from obtaining marketing authorization or that prevent or limit commercial use.

The process of obtaining marketing authorizations is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing authorization policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing authorization of a product candidate. Any marketing authorization we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. For example, the marketing authorization granted on a conditional basis by the EMA in the EEA for Translarna is limited to ambulatory nmDMD patients aged five years and older who have been identified through genetic testing and is subject to the specific obligation to conduct Study 041 and annual reassessment by the EMA of the benefit-risk analysis.

In addition, marketing authorizations in countries outside the United States do not ensure pricing approvals in those countries or in any other countries, and marketing authorizations and pricing approvals do not ensure that reimbursement will be obtained.

We may not be able to obtain orphan drug exclusivity for our products or product candidates. If our competitors are able to obtain orphan drug designations for their products and those products are determined by the FDA to be the "same drug" as our products or product candidate(s) under applicable FDA standards, or if those products can be classified as a "similar medicinal product" within the meaning of EU law, we may not be able to obtain approval by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the European Union and the United States, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan drug designations from the EMA and from the FDA for Translarna for the treatment of nmDMD and nonsense mutation aniridia. The FDA has also granted an orphan drug designation to EMFLAZA for the treatment of DMD and to RG7916, a compound under development in our SMA collaboration with Roche and the SMA Foundation. Generally, if a product with an orphan drug designation subsequently receives the first marketing authorization for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the EMA from accepting another marketing application for a similar medicinal product or the FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable market exclusivity period is currently ten years in the European Union and seven years in the United States. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is no longer justified. However, in the European Union, generic medicinal products that rely on the independently generated data submitted as part of a full marketing authorization application dossier of an authorized medicinal product, a "reference product", may not be placed on the market for 10 years from the granting of the initial marketing authorization for the reference product. In addition, the respective orphan designation and exclusivity frameworks in the United States and in the European Union are subject to change, and any such changes may affect our ability to obtain, or the impact of obtaining, EU or U.S. orphan designations in the future.

In the European Union, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. For

a drug such as Translarna, which is composed of small molecules, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use. Obtaining orphan drug exclusivity for Translarna for these indications, both in the European Union and in the United States, may be important to the product candidate's success. If a competitor obtains an orphan drug designation for and approval of a product with orphan drug exclusivity with the same indication as Translarna before we do and if the competitor's product is the same drug or a similar medicinal product as ours, we could be excluded from the market for a period of time. Even if we obtain orphan drug exclusivity for Translarna for these indications, we may not be able to maintain it. For example, if a competitive product that is the same drug or a similar medicinal product as Translarna is shown to be "clinically superior" to our product candidate as determined by the FDA, any orphan drug exclusivity we have obtained will not block the approval of such competitive product. In addition, orphan drug exclusivity will not prevent the approval of a product that is the same drug as Translarna if the FDA finds that we cannot assure the availability of sufficient quantities of the drug to meet the needs of the persons with the disease or condition for which the drug was designated. The same considerations would apply to any of our orphan product candidates.

We expect to rely on non-patent market exclusivity periods under the Hatch-Waxman Act and the Orphan Drug Act to commercialize EMFLAZA for the approved indication in the United States and failure to maintain either exclusivity period would have a material adverse effect on our ability to commercialize EMFLAZA, which in turn would have a material adverse effect on our business, financial statements and results of operations.

As we presently have no patent rights to protect the approved use of EMFLAZA, we expect to rely on non-patent market exclusivity periods under the Orphan Drug Act of 1983, or the Orphan Drug Act, and the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, to commercialize EMFLAZA in the United States.

The FDA granted an orphan drug designation to EMFLAZA for the treatment of DMD in the United States. As noted in the foregoing risk factor, generally, if a product with an orphan drug designation subsequently receives the first marketing authorization for the indication for which it has such designation, such as EMFLAZA for the treatment of DMD, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the FDA from approving another marketing application for the same drug for the same indication for that time period.

We expect that EMFLAZA will have a seven-year exclusive marketing period in the United States for the approved indication, commencing on February 9, 2017 (the date of FDA approval), under the Orphan Drug Act as well as a concurrent five-year exclusive marketing period in the United States for the active ingredient in EMFLAZA under the provisions of the Hatch-Waxman Act.

Under the Orphan Drug Act, during the seven-year exclusivity period, the FDA may not approve any other applications to market any drug considered the "same drug" as the drug with the orphan drug exclusivity for the same disease, except in limited circumstances, such as if the second applicant demonstrates the clinical superiority of its product to the product with orphan drug exclusivity through a demonstration of superior safety, superior efficacy, or a major contribution to patient care. In addition, if a company seeks orphan drug designation for a drug considered the "same drug" as a drug previously approved for the orphan indication at issue, the FDA will not designate the "same drug" as an orphan drug unless the company articulates a plausible hypothesis of the clinical superiority of its drug to the approved drug, and, following such designation, if the previously approved drug has unexpired orphan drug exclusivity, the FDA will not approve the subsequent drug unless the sponsor demonstrates clinical superiority over the previously approved drug prior to approval. As a result, in the event that a competitive product that is the "same drug" or a similar medicinal product as EMFLAZA is shown to be "clinically superior" to EMFLAZA as determined by the FDA, our orphan drug exclusivity will not block the approval of such competitive product. In addition, orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

In addition, as the holder of exclusivity, we are required to assure the availability of sufficient quantities of EMFLAZA to meet the needs of patients and failure to do so could result in loss of orphan exclusivity. Further, each of the Orphan Drug Act and the Hatch-Waxman Act is subject to change, and any such changes may affect our ability to maintain the respective market exclusivity period under those laws. Any reduction or limitation to the marketing exclusivity periods for EMFLAZA would materially limit our ability to commercialize the product, which in turn would have a material adverse effect on our business, financial statements and results of operations.

Under the Hatch-Waxman Act, a five-year period of exclusivity is granted to NDAs for products, such as EMFLAZA, containing chemical entities never previously approved by the FDA either alone or in combination. During the five-year exclusivity period, third parties may not submit certain types of applications to the FDA, except that such applications may be submitted after four years if they contain a certification of patent invalidity or non-infringement with respect to any patents of the exclusivity holder covering the drug product. The two types of applications prevented by Hatch-Waxman exclusivity are 505(b)(2) applications and abbreviated new drug applications, or ANDAs. A 505(b)(2) application allows the FDA to rely for approval of an NDA on data not developed by the applicant such as published literature or the FDA's finding of safety and effectiveness of a previously approved drug. An ANDA is an application that contains information to show that the proposed

product is identical in active ingredient, dosage form, strength, route of administration, labeling, quality, performance characteristics and intended use, among other things to a previously approved application (known as the reference listed drug). ANDAs do not contain clinical studies as required in full NDAs but are required to contain information establishing bioequivalence to the reference listed drug, allowing the FDA to use this bioequivalence information to rely on the prior finding of safety and efficacy for the reference listed drug. Exclusivity under the Hatch-Waxman Act does not prevent the submission, filing and approval of a full NDA containing full reports of investigations of safety and effectiveness either owned by the applicant or to which the applicant has obtained a right of reference. As a result, it is possible that we will not realize the full period of market exclusivity under the Hatch-Waxman Act.

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the seven-year orphan exclusivity period. This six-month exclusivity may be granted if the FDA determines that information relating to the use of the new drug in a pediatric population may produce health benefits in that population and issues a written request to the sponsor for such data, and the sponsor submits pediatric data that fairly respond to the written request within the statutory time limits. We have not received such a request from the FDA and we have not obtained pediatric exclusivity.

All pharmaceutical products for which marketing authorization has been granted, including Translarna for the treatment of nmDMD in the EEA and EMFLAZA for the treatment of DMD in the United States, are subject to extensive and rigorous governmental regulation and could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

We, Translarna, EMFLAZA, our product candidates, our operations, our facilities, our suppliers and our contract manufacturers, distributors and contract testing laboratories are subject to extensive regulation by governmental authorities in the EEA, the United States, and other territories, with regulations differing from country to country.

We are not permitted to market our product candidates in the EEA, the United States, or other territories until we have received requisite regulatory approvals. In order to receive and maintain such approvals, we and our third-party service providers must comply on a continuous basis with a broad array of regulations related to establishment registration and product listing, manufacturing processes, risk management measures, quality and pharmacovigilance systems, pre- and post-approval clinical data, labeling, advertising and promotional activities, record keeping, distribution, and import and export of pharmaceutical products for any product for which we obtain marketing authorization. Any regulatory approval of any of our products or product candidates, once obtained, may be withdrawn. For example, our marketing authorization for Translarna for the treatment of nmDMD in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, as well as the specific obligation to conduct and report the results of Study 041. In addition, we are obligated to perform certain FDA post-marketing requirements in connection with our marketing authorization for EMFLAZA in the United States, including pre-clinical and clinical safety studies. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing and distribution.

For additional information with respect to the risks related to renewal of our marketing authorization in the EEA, see the foregoing risk factor titled "*Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon our ability to satisfy the specific obligation to conduct and report the results of Study 041 by September 2021, and, as such, there is ongoing risk that we may be unable to maintain such authorization. If we are unable to obtain renewal of such marketing authorization in any future renewal cycle, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, financial performance and results of operations.*"

We are required to submit safety and other post-market information and reports, implement pharmacovigilance plans, and comply with current good manufacturing practice, or cGMP, requirements related to manufacturing including, quality control, quality assurance and complaints and corresponding maintenance of records and documents, requirements regarding the distribution of samples to healthcare professionals and recordkeeping, among other things, in connection with the marketing authorizations for Translarna for the treatment of nmDMD and for EMFLAZA for the treatment of DMD described above. Regulatory authorities, including the EMA and local regulatory authorities in EEA member states, subject a marketed product, its manufacturer and the manufacturing facilities to ongoing review and periodic inspections and the EMA is responsible for coordinating inspections, undertaken by the competent authorities of applicable member states, of our manufacturing facilities to assess whether our manufacturing, and other procedures, comply with cGMP. Similar regulatory and inspection requirements apply in other jurisdictions including those imposed by the FDA in the United States. The FDA will typically inspect a manufacturer, including its contract manufacturer organizations and clinical research organizations, following acceptance of an

NDA, which can delay FDA approval, especially if unsatisfactory inspection results are observed. If an FDA inspection were to occur and compliance issues at our facilities or at the facilities of our contract manufacturers or research organizations were identified, it could also result in disruption of production or distribution of a product or product candidate, or require substantial resources to correct.

Even if marketing authorization of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval, including the requirement of risk evaluation and mitigation strategy, or REMS. A regulatory authority also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. In addition, the competent authorities of each EU member state and the FDA closely regulate the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. Such regulatory authorities can impose stringent restrictions on our communications regarding off-label use and if we do not comply with the laws governing promotion of approved drugs, we may be subject to enforcement action for off-label promotion. For example, violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to civil and criminal penalties, investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

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

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

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

We are also subject to laws and license and registration requirements covering the distribution of marketed products. If we fail to comply with any of these requirements, we may be subject to action by regulatory agencies, which could negatively affect our business. Regulatory agencies may also change existing requirements or adopt new requirements or policies. We may be slow to adapt or may not be able to adapt to these changes or new requirements.

Commercialization of Translarna has been in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna for the treatment of nmDMD in the EEA and other countries where Translarna is

available would delay or prevent us from marketing our product in such regions, which would adversely affect our business, results of operations, and financial condition.

In some countries, particularly the member states of the EEA, the pricing of prescription pharmaceuticals is subject to strict governmental control. Each country in the EEA has its own pricing and reimbursement regulations and may have other regulations related to the marketing and sale of pharmaceutical products in the country. We generally will not be able to commence commercial sales of Translarna for the treatment of nmDMD pursuant to the conditional marketing authorization granted by the European Commission in any particular member state of the EEA until we conclude the applicable pricing and reimbursement negotiations and comply with any licensing, employment or related regulatory requirements in that country. In some countries we may be required to conduct additional clinical trials or other studies of our product, including trials that compare the cost-effectiveness of our product to other available therapies in order to obtain reimbursement or pricing approval. We may not be able to conclude pricing and reimbursement negotiations or comply with additional regulatory requirements in the countries in which we seek to commercialize Translarna on a timely basis, or at all.

The pricing and reimbursement process varies from country to country and can take over 18 months to complete. Pricing negotiations may continue after reimbursement has been obtained. We cannot predict the timing of Translarna's commercial launch in countries where we are awaiting pricing and reimbursement guidelines. While we have submitted pricing and reimbursement dossiers with respect to Translarna for the treatment of nmDMD in many EEA countries, we have only received both pricing and reimbursement approval on terms that are acceptable to us in a limited number of countries.

The price that is approved by governmental authorities in any country pursuant to commercial pricing and reimbursement processes may be significantly lower than the price we are able to charge for sales under our reimbursed EAP programs. In some instances, reimbursement may be subject to challenge, reduction or denial by the government and other payors.

For example, in France, EAP and commercial sales of a product can begin while pricing and reimbursement rates are under discussion with the applicable government health programs. In the event that the negotiated price of the product is lower than the amount reimbursed for sales made prior to the conclusion of price negotiations, we may become obligated to repay such excess amount to the applicable government health program. We will make such retroactive reimbursement, if any, following the conclusion of price negotiations with the applicable government health authority.

Further, based on unsustainable economics imposed by the arbitration board in Germany upon the conclusion of an arbitration process in 2016 with us and the German Federal Association of the Statutory Health Insurances, we delisted Translarna from the German pharmacy ordering system, effective April 1, 2016. While some patients and healthcare professionals in Germany have been able to access Translarna through a reimbursed importation pathway possible under German law, there can be no assurance that other patients or healthcare professionals in Germany will be successful doing so or, if initially successful, that any or all will continue to be successful. We were required to reimburse payors in Germany the difference between the commercial price of Translarna and the price established by the arbitration board in Germany for sales made in Germany after December 2015, other than sales made pursuant to the reimbursed importation pathway.

Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations and there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. For example, these factors influenced the length of our pricing and reimbursement negotiations in England, which took place between mid-2014 to mid-2016, and culminated in a five-year managed access agreement between us, National Health Services England, the National Institute for Health and Care Excellence, or NICE, NorthStar clinical network and the patient organizations Muscular Dystrophy UK and Action Duchenne. The managed access agreement establishes the clinical details surrounding the use of Translarna, including the terms and conditions of a confidential financial arrangement and the collection of further data on the efficacy of Translarna for the treatment of nmDMD with NICE guidance to be reviewed again at the end of the five-year period, before future funding decisions are taken.

In addition, adverse clinical and regulatory developments may exacerbate these risks, including the developments noted in the foregoing risk factor titled, "*ACT DMD did not meet its primary efficacy endpoint, and there is substantial risk that regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials in Translarna for the treatment of nmDMD, which would have a material adverse effect on our business, financial performance and results of operations.*"

Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices and revenues. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries.

If we fail to successfully secure and maintain pricing and reimbursement coverage for Translarna or are significantly delayed in doing so or if burdensome conditions are imposed by private payers, government authorities or other third-party payers on such

reimbursement, planned launches in the affected countries will be delayed and our business, results of operations and financial condition could be adversely affected.

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

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any products or product candidates, including Translarna and EMFLAZA, for which we have obtained or may obtain marketing approval. Our arrangements with customers, healthcare providers and professionals and third-party payors may expose us to broadly applicable fraud and abuse, transparency and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing authorization.

Failure to maintain a comprehensive and effective compliance program, and to integrate the operations of any acquired businesses into a combined comprehensive and effective compliance program on a timely basis, could subject us to a range of regulatory actions that could adversely affect our ability to commercialize our products and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products.

Restrictions and reporting requirements under applicable U.S. federal and state healthcare laws and regulations, and equivalent laws and regulations in the European Union and other countries in which we operate, include, and are not limited to, the following:

- Anti-corruption and anti-bribery laws and regulations, such as the U.S. Foreign Corrupt Practices Act, or FCPA, the UK Bribery Act of 2010, or Bribery Act, and similar statutes which have been adopted, or may be adopted in the future, by other countries in which we operate and with which we are or may be required to comply.

- Anti-kickback laws and regulations, including those applicable in the United States, the United Kingdom and other countries where we operate, which generally prohibit, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under government funded healthcare programs. The U.S. federal statute imposes criminal penalties and has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others and many states have enacted equivalent state laws that apply not only to government payors but to commercial payors as well.

- False claim laws and regulations, including the U.S. False Claims Act and similar state laws, which may permit civil whistleblower or qui tam actions and may impose civil liability and criminal penalties on individuals and entities who submit, or cause to be submitted, false or fraudulent claims for payment to the government. Federal enforcement agencies have also showed increased interest in pharmaceutical companies' product and patient assistance programs, including reimbursement and co-pay support services, and a number of investigations into these programs have resulted in significant civil and criminal settlements.

- Laws and regulations related to the privacy, security and transmission of individually identifiable health information, including the U.S. Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and similar state laws, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information, and may impose criminal and civil liability for violations of these obligations. In addition, international data protection laws including the European Union Data Protection Directive and member state implementing legislation may apply to some or all of the clinical data obtained, transmitted, or stored outside of the United States. Furthermore, certain privacy laws and genetic testing laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our use and dissemination of individuals' health information.

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

- Laws and regulations governing the advertising and promotion of medicinal products, interactions with physicians and patients, misleading and comparative advertising and unfair commercial practices. For example, legislation adopted by individual EU member states that may apply to the advertising and promotion of medicinal products require that

promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, as approved by the competent authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. Promotion of indications not covered by the SmPC is specifically prohibited.

- Laws and regulations regulating off-label promotion of medicinal products, which is prohibited in the European Union. The applicable laws at European Union level and in the individual EU member states also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the European Union could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on our promotional activities with health care professionals.

- Laws and regulations in the United States, including the Federal Food, Drug and Cosmetic Act and other laws and regulations, that prohibit us from promoting any of our FDA approved products for off-label uses. This means, for example, that we cannot make claims about the use of our marketed products or their relative benefits compared to other treatments outside of their FDA approved indications and label, and we would not be able to proactively discuss or provide information on off-label uses or safety benefits of such products, with very specific and limited exceptions. Should the FDA determine that our activities constituted the promotion of off-label use, the FDA could bring action to prevent us from distributing those products for the off-label use and could impose fines and penalties on us and our executives, and such a determination could also trigger civil or criminal liability under other applicable laws in the United States.

- Laws and regulations requiring that we disclose publicly payments made to physicians, including in certain EU member states and the United States. For example, in the United States, under the federal Physician Payments Sunshine Act requirements, manufacturers of drugs, devices, biologics and medical supplies must report information related to payments and other transfers of value made to or at the request of covered recipients, such as physicians and teaching hospitals, as well as physician ownership and investment interests in such manufacturers. A number of U.S. states and other countries have enacted their own transparency requirements that obligate manufacturers to report different types of spending related to physicians, certain hospitals, and other covered recipients.

In addition, interactions between pharmaceutical companies and physicians are also governed by industry self-regulation codes of conduct and physicians' codes of professional conduct. In the United States, some state laws require pharmaceutical companies to comply with these industry and physician codes and the relevant compliance guidance promulgated by the federal government. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national laws of the EU member states, as well as codes of conduct issued by self-regulatory industry bodies. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, their competent professional organization, and the competent authorities of the individual EU member states. These requirements are provided in the national laws, industry codes, or professional codes of conduct, applicable in the EU member states.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws, regulations, transparency requirements and self-regulatory codes have and will continue to involve substantial costs. We cannot guarantee that we, our employees, our consultants, our third-party contractors, or the physicians or other providers or entities with whom we expect to do business, are or will be in compliance with all federal, state and foreign regulations and codes. It is possible that governmental authorities could conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, and the curtailment or restructuring of our operations. Exclusion, suspension and debarment from government funded healthcare programs would adversely affect, perhaps materially, our ability to commercialize, sell or distribute any drug. Even if we were not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could also have an adverse effect on our business, financial condition and results of operations.

Legislative and regulatory changes affecting the pharmaceutical industry or the healthcare system more broadly may increase the difficulty and cost for us to obtain or maintain marketing authorization of and commercialize our products and product candidates and affect the coverage and reimbursement we may obtain.

Our industry is highly regulated and changes in law may adversely impact our business, operations, or financial results. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed

changes regarding the healthcare system that could prevent or delay marketing authorization of Translarna or any of our other product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products or product candidates, including Translarna and EMFLAZA, for which we have obtained, or may obtain, marketing authorization.

Certain provisions of enacted or proposed legislative changes may negatively impact coverage and reimbursement of healthcare items and services. For example, in the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and reimbursement that we receive for any approved products, or allow for the importation of lower-priced versions of our approved products from Canada. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own policies. Therefore, any restrictions to coverage or reductions in reimbursement that result from the Medicare Modernization Act may result in a similar coverage restriction or reimbursement reduction from private payors. In addition, private payors may implement coverage restrictions or payment reductions independently from federal programs such as Medicare.

Similarly, in the United States, the Affordable Care Act contains provisions that may reduce the profitability of drug products. However, the new Presidential Administration and U.S. Congress have expressed a desire to modify, repeal or otherwise invalidate all, or certain provisions of, the Affordable Care Act, which has contributed to the uncertainty of the ongoing implementation and impact of the Affordable Care Act and also underscores the potential for additional reform going forward. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results.

We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

- controls on government funded reimbursement for drugs;
- caps on mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits on prohibitions on reimbursement or specific products through other means;
- reform of drug importation laws;
- expansion of use of managed care systems in which the healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third-party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for our products, which would have an adverse effect on our net revenues and operating results.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize Translarna and our product candidates. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. We cannot predict how future changes relating to healthcare reform in the European Union, the United States, or other territories, will affect our business.

Legislative and regulatory proposals have also been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative or regulatory changes will be enacted in any territory in which we are authorized, or become authorized, to market Translarna, EMFLAZA, or any of our other product candidates, or whether applicable regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing authorizations of our products or product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process or by comparable foreign bodies overseeing regulatory authorities in other territories may significantly delay or prevent marketing authorization, as well as subject us to more stringent product

labeling and post-marketing testing and other requirements. We cannot predict how future changes relating to pre- and post-marketing approval and requirements will affect our business.

Risks Related to our Dependence on Third Parties

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

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

We currently rely on a single source for the production of some of our raw materials and we obtain our supply of the drug substance for Translarna from two third-party manufacturers and the drug substance for our cancer stem cell program through another third-party manufacturer. We engage two separate manufacturers to provide bulk drug product for Translarna. We have a relationship with three manufacturers that are capable of providing fill and finish services for our finished commercial and clinical Translarna product, although we are still in the process of finalizing arrangements with one of these manufacturers with respect to commercial product services. We anticipate completing applicable validation procedures for this manufacturer in 2017 for both commercial and clinical product.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of Translarna or any of our product candidates, although we may seek to establish such arrangements in the future. In the event that we are unable to procure supply from a validated manufacturer, we would seek to identify and qualify replacement suppliers, however this process would likely result in delays in our ability to supply Translarna to patients or advance our clinical trials. We may be unable to conclude agreements for commercial or clinical supply of Translarna with third-party manufacturers, or may be unable to do so on acceptable terms.

We currently have a contract with a pharmacy and hospital distributor in the European Union that distributes Translarna for clinical programs and limited commercial and EAP programs. We have engaged with third-party logistic providers, or 3PLs, which distribute Translarna for the majority of our commercial and EAP programs on our behalf. We intend to engage additional distributors if and when, if ever, we become authorized to make Translarna available for purchase in such additional geographies.

We obtain our supply of the drug substance for EMFLAZA through a third-party manufacturer that is currently the only third-party manufacturer qualified to provide EMFLAZA drug substance. All of our drug product manufacturing, processing and packaging needs for EMFLAZA tablet and suspension product will be fulfilled pursuant to two different exclusive supply agreements assumed by us in connection with our acquisition of EMFLAZA. We expect to fulfill all of our requirements for EMFLAZA tablets as well as secondary packaging of pre-filled EMFLAZA oral suspension bottles pursuant to one of these agreements, which has an initial term of five years. We expect to fulfill all of our requirements for EMFLAZA suspension product pursuant to the other agreement. Through the seventh year anniversary of FDA approval of EMFLAZA, we are obligated to pay to the manufacturer of the EMFLAZA suspension product royalty payments, on a quarterly basis, based on a percentage (ranging from low to middle-low double digits) of, or a fixed payment with respect to, our annual net sales of suspension product in the United States, subject to reduction in accordance with the terms of the agreement. The royalty payments for the suspension product are subject to a minimum aggregate annual payment ranging from €0.5 million to €1.5 million per year.

If our drug substance provider or either of our drug product manufacturers was to be unable to provide drug substance or manufacture EMFLAZA product in sufficient quantities to meet projected demand, future sales could be adversely affected, which in turn could have a detrimental impact on our ability to maintain our marketing authorization in the United States and on our ability to commercialize EMFLAZA, which in turn would have a material adverse effect on our business, financial results and results of operations. Further, as we presently have no patent rights to protect the approved use of EMFLAZA, we expect to rely market exclusivity periods available to us under the Orphan Drug Act and Hatch-Waxman Act to commercialize EMFLAZA for DMD in the United States. As the holder of orphan exclusivity, we are required to assure the availability of sufficient quantities of EMFLAZA to meet the needs of patients. Failure to do so could result in loss of the drug's orphan exclusivity in the United States, which would have a material adverse effect on our ability to generate revenue from sales of EMFLAZA.

We utilize third parties for the commercial distribution of EMFLAZA, including a 3PL to warehouse EMFLAZA as well as specialty pharmacies to sell and distribute EMFLAZA to patients. A specialty pharmacy provides us with third-party call center services to provide patient support and financial services, prescription intake and distribution, reimbursement adjudication, and ongoing compliance support. If we are unable to effectively manage this distribution process, the continuance of our commercial launch and sales of EMFLAZA may be delayed or compromised.

Even if we are able to establish and maintain arrangements with third-party manufacturers and distributors, reliance on such service providers as well as the use of specialty pharmacies and a call center entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possibility of commercial supplies of Translarna or EMFLAZA not being distributed to commercial vendors or end users in a timely manner, resulting in lost sales;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions;
- the possibility of third-party resources not being devoted in the proper manner necessary to satisfy our requests and needs within the time frame we expect;
- the possibility of third parties not providing us with accurate or timely information regarding their inventories, the number of patients who are using EMFLAZA, or serious adverse events and/or product complaints regarding EMFLAZA;
- the third parties being unable to satisfy their financial obligations to us or others; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Many additional factors could cause production or distribution interruptions with the manufacture and distribution of Translarna or EMFLAZA and any of our product candidates, including human error, natural disasters, labor disputes, acts of terrorism or war, equipment malfunctions, contamination, or raw material shortages.

In addition, third-party manufacturers or distributors may not be able to comply with current good manufacturing practice, or cGMP, or good distribution practice, or GDP, or similar regulatory requirements outside the European Union and the United States. Our failure, or the failure of our third-party manufacturers or distributors, over whom we have no control, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or product, operating restrictions, criminal prosecutions or debarment, any of which could significantly and adversely affect supplies of Translarna, EMFLAZA or our product candidates.

Our products and our product candidates and any other products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our products and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively.

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

Our current and anticipated future dependence upon others for the manufacture and distribution of Translarna, EMFLAZA and our product candidates may adversely affect our business, financial condition, results of operations and limit our ability to grow including our ability to develop product candidates and commercialize our products that receive regulatory approval on a timely and competitive basis.

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

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

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

For example, in the first half of 2013 inspectors acting at the request of the EMA conducted GCP inspections of selected clinical sites from our completed Phase 2b clinical trial of Translarna for the treatment of nmDMD and our clinical trial site relating to our then pending marketing authorization application for approval of Translarna for the treatment of nmDMD. Following these inspections, we received inspection reports containing a combination of critical and major findings. These findings related to waivers we granted to admit patients to our Phase 2b clinical trial of Translarna for the treatment of nmDMD in advance of formal approval of protocol amendments that would have established their eligibility for the trial, as well as our oversight of our trial sites and the completeness or sufficiency of clinical trial documentation. In response to these findings, we described to the EMA the enhanced internal procedures and controls we have implemented, and the internal quality assurance department we have established, since the conclusion of our Phase 2b clinical trial of Translarna for the treatment of nmDMD. In addition, we proposed corrective action plans to address the inspectors' specific findings. If we do not meet our commitment to the corrective actions we proposed to the EMA, we may face additional consequences, including rejection of data or other direct action by national regulatory authorities, which could require us to conduct additional clinical trials or other supportive studies to maintain our marketing authorization in the EEA for Translarna for the treatment of nmDMD or to obtain full approval from the EMA.

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

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

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

For each of our product candidates, we plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaborations with Roche and the SMA Foundation, for our spinal muscular atrophy program. We have entered into arrangements with certain third parties to market or distribute Translarna for the treatment of nmDMD in certain countries and, as we continue to implement our commercialization plans for Translarna, we anticipate that we will engage additional third parties to perform these functions for us in other countries. We generally plan to seek collaborators for the development and commercialization of product candidates that have high anticipated development costs, are directed at indications for which a potential collaborator has a particular expertise, or involve markets that require a large sales and marketing organization to serve effectively. Our likely collaborators for any marketing, distribution, development, licensing or broader collaboration arrangements may include: large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and/or biotechnology companies.

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;
- we may grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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

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

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

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

pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

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

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

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

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

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

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

Risks Related to our Intellectual Property

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

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

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

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, patent law in many countries restricts the patentability of methods of treatment of the human body more than U.S. law does. In addition, we may not pursue or obtain or be able to pursue or obtain patent protection in all major markets. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. In addition, the Leahy-Smith America Invents Act of 2011 (the “Act”), which reformed certain patent laws in the U.S., may create additional uncertainty. The significant changes engendered by the Act include switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the USPTO after grant, including inter partes review and post grant review.

Moreover, we may be subject to a third party anonymously submitting prior art to a patent office or may become involved in addressing patentability objections based on third-party submission of references, or may become involved in oppositions, derivation proceedings, reexamination, inter partes review, post grant review, interference proceedings or other patent office proceedings or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize our product or current or future product candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. In addition, other companies may attempt to circumvent any regulatory data protection or market exclusivity that we obtain under applicable legislation, which may require us to allocate significant resources to preventing such circumvention. Legal and regulatory developments in the European Union and elsewhere may also result in clinical trial data and other information, that would ordinarily be treated as trade secret, submitted as part of a marketing authorization application becoming publicly available. The EMA Policy on publication of clinical data and other such information, as well as the current application of European Union freedom of information regulations, could impact our proprietary information (comprising both clinical and non-clinical data and other information) that would normally be maintained by a regulatory body as commercially confidential. Such developments could enable other companies to circumvent our intellectual property rights and use our clinical trial data or other information to obtain marketing authorizations in the European Union and in other jurisdictions. Such developments may also require us to allocate significant resources or engage in litigation to prevent other companies from circumventing or violating our intellectual property rights. Our attempts to prevent third parties from circumventing our intellectual property and other rights may ultimately be unsuccessful. We may also fail to take the required actions or pay the necessary fees to maintain our patents.

For example, during 2015, we were notified by the EMA that it had received from another pharmaceutical company a request under Regulation (EC) No 1049/2001 seeking access to aspects of our marketing authorization for Translarna for the treatment of nmDMD. Following the decision of the EMA to release such documentation with only minimal redactions we initiated litigation before the General Court of the European Union to prevent disclosure of this information, and in July 2016, the Court took the interim measure of ordering the EMA not to release our documents until the substantive case has been decided (by the General Court and/or in possible appeal proceedings). The EMA appealed the interim measure to the Court of Justice of the European Union but the Court of Justice dismissed the appeal in the first quarter of 2017. While we expect to continue this litigation to a final decision on its underlying merits, as well as to object to the disclosure of any information that we consider commercially confidential, there can be no assurance that we will be successful in the aforementioned litigation or in any future challenge that may be raised and we may not ultimately be successful in preventing disclosure of the data in our marketing authorization for Translarna for the treatment of nmDMD.

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

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

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

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our products and our product candidates and use our proprietary technologies without infringing the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, inter partes review or post-grant review proceedings before the U.S. Patent and Trademark Office. The risks of being involved in such litigation and proceedings may also increase as our product candidates approach commercialization, and as we gain greater visibility as a public company. Third parties may assert infringement claims against us based on existing or future intellectual property rights. We may not be aware of all such intellectual property rights potentially relating to our product and our product candidates. Since patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, with new publications occurring continuously, there may be patents or patent applications relating to our product or our product candidates that we are unaware of. There may also be pending or future patent applications that, if issued, would block us from commercializing Translarna or EMFLAZA. Thus, we do not know with certainty whether Translarna, EMFLAZA, or any of our other product candidates, or our commercialization thereof, would or would not infringe any third party's intellectual property.

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

materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

For example, it is possible that one or more third parties might bring a patent infringement or other legal proceeding against us regarding Translarna or EMFLAZA. We are aware of an issued U.S. patent and international patent applications that purport to disclose or contain claims to chemical scaffolds that are sufficiently broad that they could be read to encompass ataluren, the active ingredient in Translarna, even though neither the issued U.S. patent nor any of the international patents or patent applications specifically discloses ataluren. In order to successfully challenge the validity of any issued U.S. patent that may allegedly include ataluren within the scope of a granted claim, we would need to overcome that patent's presumption of validity in district court or prove unpatentability by a preponderance of the evidence before the USPTO. There is no assurance that a court or the USPTO would find these claims to be invalid or unpatentable, respectively. In addition, we believe that the public notice given by our testing of ataluren in clinical trials for the purpose of seeking FDA approval would be a valid defense against any infringement claims in the United States based on the availability of any statutory research exemptions. However, there can be no assurance that our interpretation of the exemption would be upheld, were the exemption interpreted as covering only our preclinical research activities, and not the commercialization of ataluren.

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

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

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

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

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

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

Without patent protection, our marketed products may face generic competition.

Certain of the products we market have no patent protection and, as a result, potential competitors face fewer regulatory barriers in introducing competing products. Without patent protection or other regulatory exclusivity, we may not be able to exclude others from, among other things, selling or importing similar products in any jurisdiction. In some instances, we may rely on trade secrets and other unpatented proprietary information to protect our commercial position with respect to such products, although we may be unable to provide adequate protection for our commercial position via these means. In other instances, we may need to rely on regulatory exclusivity to protect our commercial position.

Furthermore, generic competition against a branded product often results in decreases in the prices at which the branded product can be sold, particularly when there is more than one generic product available in the marketplace. Third-party companies could also develop products that are similar, but not identical, to our marketed products, such as an alternative formulation of our product or an alternative formulation combined with a different delivery technology, and seek approval in

the United States by referencing our products and relying, to some degree, on the FDA's finding that our products are safe and effective in their approved indications. In addition, legislation enacted in the United States allows for, and in a few instances, in the absence of specific instructions from the prescribing physician, mandates the dispensing of generic products rather than branded products where a generic version is available.

On February 9, 2017, the FDA approved the corticosteroid EMFLAZA (deflazacort) for the treatment of patients 5 years and older with DMD. Although approved for other indications outside of the United States, this was the first approval for deflazacort in the United States and the first approval in the United States for the use of a corticosteroid to treat DMD.

We rely on regulatory exclusivity for EMFLAZA and currently have no issued patents that could prevent a third-party company from seeking to introduce a generic EMFLAZA formulation in the United States for the treatment of DMD or another indication, and we may never be able to obtain such patent protection. Such third-party companies may also obtain patents covering a new deflazacort formulation or method of use, and attempt to assert such patents against us.

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

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

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

Our trademark applications may be refused registration, and our registered trademarks may not be maintained or may be found to be unenforceable. During trademark examination proceedings, our trademark applications may be rejected. Although we are given an opportunity to respond to those rejections, we may not be able to overcome them. In addition, in the U.S. Patent and Trademark Office and Trademark Offices in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications or to seek cancellation of registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. In addition, if we do not secure registrations for our trademarks, we may encounter difficulty enforcing our trademark rights against third parties in the jurisdictions where we do not have registered rights.

If we are not able to obtain adequate trademark protection or regulatory approval for our brand names, including Translarna and EMFLAZA, we may be required to re-brand affected products, which could cause delays in getting such products to market and substantially increase our costs.

To protect our rights in any trademark we intend to use for our products or our product candidates, including Translarna and EMFLAZA, we may seek to register such trademarks. Trademark registration is territory-specific and we must apply for trademark registration in the United States as well as any other country where we intend to commercialize our product or product candidates. Failure to obtain trademark registrations may place our use of the trademarks at risk or make them subject to legal challenges, which could force us to choose alternative names for our product or product candidates. In addition, the

FDA, and other regulatory authorities outside the United States, conduct an independent review of proposed product names for pharmaceuticals, including an evaluation of the potential for confusion with other pharmaceutical product names for medications, which could result in medication errors in prescribing, dispensing and consumption. These regulatory authorities may also object to a proposed product name if they believe the name inappropriately makes or implies a therapeutic claim. If the FDA or other regulatory authorities outside the United States object to any of our proposed product names, we may be required to adopt alternative names for our product or product candidates. If we adopt alternative names, either because of our inability to obtain a trademark registration or because of objections from regulatory authorities, we would lose the benefit of our existing trademark applications and the rights attached thereto. Consequently, we may be required to expend significant additional resources in an effort to adopt a new product name that would be registrable under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA and other regulatory authorities, which could cause delays in getting our products to market and substantially increase our costs. Furthermore, we may not be able to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product or our product candidates.

Risks Related to Employee Matters and Managing Growth

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

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

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

We are in the process of expanding our development, regulatory, and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

In connection with our commercialization plans and business strategy, including our commercial launch of Translarna for the treatment of nmDMD and our integration of and commercialization efforts related to EMFLAZA, we have experienced and may to continue to experience significant growth in our employee base for sales, marketing, operational, managerial, financial, human resources, drug development, quality, regulatory and medical affairs and other areas. This growth has imposed and will continue to impose significant added responsibilities on members of management, including the need to recruit, hire, retain, motivate and integrate additional employees, including employees who joined us in connection with our acquisition of EMFLAZA. Also, our management may have to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities, including the integration of EMFLAZA. To manage our recent and anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. In addition, we may need to adjust the size of our workforce as a result of changes to our expectations for our business, which can result in diversion of management attention, disruptions to our business, and related expenses. For example, following our receipt of the Refuse to File letter from the FDA in 2016, we implemented a reorganization of our operations in March 2016 that resulted in a one-time charge for the related work-force reduction. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to our Common Stock

Servicing the Convertible Notes will require a significant amount of cash. We may not have sufficient cash flow from our business to make payments on our debt, and we may not have the ability to raise the funds necessary to settle conversions of,

or to repurchase, the Convertible Notes upon a fundamental change, which could adversely affect our business, financial condition and results of operations.

In August 2015, we incurred indebtedness in the amount of $150.0 million in aggregate principal with additional accrued interest under the Convertible Notes, for which interest is payable semi-annually in arrears on February 15 and August 15 of each year, beginning on February 15, 2016. Our ability to make scheduled payments of the principal of, to pay interest on or to refinance the Convertible Notes depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service our debt, including the Convertible Notes. If we are unable to generate cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be unfavorable to us or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at the time we seek to refinance such indebtedness. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

In addition, upon conversion of the Convertible Notes unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional shares), we will be required to make cash payments in respect of the Convertible Notes being converted. However, we may not have enough available cash or be able to obtain financing at the time we are required to repurchase Convertible Notes, to pay the Convertible Notes at maturity or to pay cash upon conversions of Convertible Notes. In addition, our ability to repurchase Convertible Notes or to pay cash upon conversions of Convertible Notes may be limited by law, by regulatory authority or by agreements governing our future indebtedness. Our failure to repurchase Convertible Notes at a time when the repurchase is required by the indenture, to make interest payments on the Convertible Notes when due under the indenture or to pay any cash payable on future conversions of the Convertible Notes as required by the indenture would constitute a default under the indenture. An event of default under the indenture governing the Convertible Notes or the fundamental change itself could also lead to a default under agreements governing our future indebtedness. If the repayment of any such related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness, repurchase the Convertible Notes, make interest payments on the Convertible Notes or make cash payments upon conversions of the Convertible Notes.

In addition, even if holders of the Convertible Notes do not elect to convert their Convertible Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the Convertible Notes as a current rather than long-term liability, which would result in a material reduction of our net working capital. Any of these factors could materially and adversely affect our business, financial condition and results of operations.

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

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

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

- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

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

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock and lawsuits against us and our officers and directors.

Our stock price has been and will likely continue to be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- any developments related to our ability or inability to execute our strategy for EMFLAZA for the treatment of DMD in the United States, in particular with respect to our commercialization efforts;
- any developments related to our ability or inability to advance Translarna for the treatment of nmDMD in the United States in a timely manner or at all, whether pursuant to the file over protest process with the FDA, or otherwise, and including whether we will be required to complete any additional clinical and non-clinical trials or analyses;
- our ability to maintain our marketing authorization for Translarna for the treatment of nmDMD in the EEA, which is subject to the specific obligation to conduct Study 041 and is also subject to annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;
- any developments related to Study 041, including with respect to design, timing, conduct, and enrollment, and developments with respect to any clinical or non-clinical trial that may be required by other regulatory agencies, including the FDA for Translarna for the treatment of nmDMD;
- results of clinical trials of Translarna and any other product candidate that we develop;
- announcements by us or our competitors of significant acquisitions, licenses, strategic collaborations, joint ventures, collaborations or capital commitments;
- negative publicity around our products or product candidates, including with respect to EMFLAZA;
- other developments concerning our regulatory submissions;
- whether regulators in other territories agree with our interpretation of the results of ACT DMD;
- our ability to advance the commercialization of Translarna for the treatment of nmDMD;
- the success of competitive products or technologies;
- the development and regulatory status of our SMA program with Roche and the SMA Foundation;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our products, product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;

- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

Companies that have experienced volatility in the market price of their stock have frequently been the subject of securities class action and shareholder derivative litigation. See Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q for information concerning litigation initiated against us and certain of our officers during the first quarter of 2016. In addition, we could be the target of other such litigation in the future. Class action and derivative lawsuits, whether successful or not, could result in substantial costs, damage or settlement awards and a diversion of our management's resources and attention from running our business, which could materially harm our reputation, financial condition and results of operations.

We are currently incurring and expect to continue to incur increased costs as a result of operating as a public company, including compliance with Section 404 of the Sarbanes-Oxley Act of 2002, and our management is and will continue to be required to devote substantial time to compliance initiatives. In addition, the failure to establish and maintain adequate finance infrastructure and accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Act, the listing requirements of The NASDAQ Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel have and will need to continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have and will continue to increase our legal and financial compliance costs and will continue to make some activities more time-consuming and costly. For example, these rules and regulations have made it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on the effectiveness of our internal control over financial reporting and an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. Compliance with Section 404, including documentation and evaluation of our internal control over financial reporting, is both costly and challenging. If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner each year, we could be subject to sanctions or investigations by the Securities and Exchange Commission, the NASDAQ Stock Market or other regulatory authorities which would require additional financial and management resources and could adversely affect the market price of our common stock. Furthermore, if we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information.

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

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

Sales of a substantial number of shares of our common stock in the public market by our existing stockholders could significantly reduce the market price of our common stock.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

We have issued a significant number of equity awards under our equity compensation plans or as inducement grants to new hire employees pursuant to Nasdaq rules. The shares underlying these awards are or, with respect to certain option grants, will be registered on a Form S-8 registration statement. As a result, upon vesting these shares can be freely exercised and sold in the public market upon issuance, subject to volume limitations applicable to affiliates. The exercise of options and the subsequent sale of the underlying common stock or the sale of restricted stock upon vesting could cause a decline in our stock price. These sales also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Certain of our employees, executive officers and directors have entered or may enter into Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the employee, director or officer when entering into the plan, without further direction from the employee,

officer or director. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers and directors may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

In connection with our acquisition of EMFLAZA, we issued an affiliate of Marathon 6,683,598 shares of our common stock. While these shares are currently restricted as a result of securities laws, following expiration of applicable holding periods, the shares will be able to be freely sold in the public market subject to any requirements and restrictions, including any applicable volume limitations, imposed by Rule 144 under the Securities Act. The sale or resale of these shares in the public market, or the market's expectation of such sales, may result in an immediate and substantial decline in our stock price. Such a decline will adversely affect our investors and also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Securities

Inducement stock option awards. Pursuant to the NASDAQ inducement grant exception, during the quarter ended June 30, 2017, we issued options to purchase an aggregate of 425,650 shares of common stock to certain new hire employees at a weighted-average exercise price of $12.99 per share. The shares underlying these option awards will be registered on a Form S-8 registration statement prior to the first vesting event applicable to each such award.

Item 6. Exhibits.

Those exhibits required to be filed by Item 601 of Regulation S-K are listed in the Exhibit Index immediately preceding the exhibits hereto and such listing is incorporated herein by reference.

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: August 9, 2017

By: /s/ Christine Utter

Christine Utter

Principal Financial Officer

(Principal Financial and Accounting Officer and Duly Authorized Signatory)

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
2.1†	Asset Purchase Agreement, dated March 15, 2017, between PTC Therapeutics, Inc. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC) (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on March 16, 2017)
2.2	Amendment to Asset Purchase Agreement, dated April 20, 2017, between PTC Therapeutics, Inc. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC) (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on April 20, 2017)
3.1	Amended and Restated Bylaws (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K filed by the Registrant on April 21, 2017)
10.1††	Exclusive License and Supply Agreement, dated as of May 12, 2015, as amended, by and between Faes Farma, S.A. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC), as assigned by Complete Pharma Holdings, LLC to the Registrant on April 20, 2017
10.2††	Commercial Manufacturing Agreement, dated as of September 18, 2015, as amended, by and between Alcami Corporation (f/k/a/ AAI Pharma Services Corp.) and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC), as assigned by Complete Pharma Holdings, LLC to the Registrant on April 20, 2017
10.3	Credit and Security Agreement, dated May 5, 2017, by and among PTC Therapeutics Inc., MidCap Financial Trust and the additional lenders thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on May 8, 2017)
10.4	Pledge Agreement, dated May 5, 2017, by and among PTC Therapeutics Inc., each of the subsidiaries listed thereto as pledgers and MidCap Financial Trust (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed by the Registrant on May 8, 2017)
10.5	Intellectual Property Security Agreement, dated May 5, 2017, by and among PTC Therapeutics Inc. and MidCap Financial Trust (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed by the Registrant on May 8, 2017)
10.6+	Employment Agreement, as amended, between the Registrant and Marcio Souza
10.7+	Employment Agreement, as amended, between the Registrant and Christine Utter
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	XBRL Instance Document*
101.SCH	XBRL Taxonomy Extension Schema Document*
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document*
101.LAB	XBRL Taxonomy Extension Label Linkbase Database*
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document*
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document*

* Submitted electronically herewith.

† Confidential treatment has been granted for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the U.S. Securities and Exchange Commission (the "SEC") pursuant to the registrant's application for confidential treatment. In addition, schedules have been omitted from this exhibit pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule will be furnished supplementally to the SEC upon request; provided, however, that the registrant may request confidential treatment for any document so furnished.

†† Confidential treatment has been requested for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the U.S. Securities and Exchange Commission (the "SEC") pursuant to the registrant's application for confidential treatment. In addition, schedules have been omitted from this exhibit pursuant to Item 601(b)(2) of

Regulation S-K. A copy of any omitted schedule will be furnished supplementally to the SEC upon request; provided, however, that the registrant may request confidential treatment for any document so furnished.

+ 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.

Exhibit 10.1

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

EXCLUSIVE LICENSE AND SUPPLY AGREEMENT

THIS EXCLUSIVE LICENSE AND SUPPLY AGREEMENT (this "**Agreement**") is made and entered into as of May 12, 2015 (the "**Effective Date**"), by and between Faes Farma, S.A., a corporation (*sociedad anónima*) organized under the Laws of Spain and having offices located at Avenida Autonomía 10, 48.940 Leioa (Biscay) Spain ("**Faes**"), and Marathon Pharmaceuticals, LLC, a limited liability company organized under the Laws of the State of Delaware (U.S.A.) and having offices located at 1033 Skokie Boulevard, Suite 600, Northbrook, Illinois 60062 U.S.A. ("**Marathon**"). Faes and Marathon are sometimes individually referred to herein as a "**Party**" and collectively as the "**Parties**".

WHEREAS, Faes owns the Faes Product and owns or controls (whether by license or otherwise) the Licensed Assets.

WHEREAS, Marathon is conducting research and development with respect to the Marathon Tablet Product in and for the Territory.

WHEREAS, the Parties desire to enter into this Agreement, pursuant to which, among other things, (i) Faes shall grant to Marathon a license in, to and under the Faes Product and the Licensed Assets to research, Develop, obtain regulatory approval for, market, promote, distribute, sell, use, commercialize and, solely as expressly permitted under this Agreement, manufacture (or have manufactured by an Affiliate, subsidiary or Third Party) the Marathon Suspension Product in and for the Territory, as a complement to the Marathon Tablet Product, (ii) Marathon shall (except as expressly provided for under this Agreement) purchase exclusively from Faes all of its requirements for Marathon Suspension Product for the Territory during the Exclusive Manufacturing Term, and (iii) Marathon shall pay Royalties to Faes during the Royalty Term, on and subject to the terms and conditions set forth herein.

NOW, THEREFORE, for and in consideration of the covenants, conditions and undertakings set forth herein and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, on and subject to the terms and conditions hereof, the Parties, intending legally to be bound hereby, agree as follows:

Section 1

Definitions; Interpretation

1.1 Definitions. Capitalized terms used, but not otherwise defined, in this Agreement have the following meanings:

"**Act**" shall mean the United States Federal Food, Drug and Cosmetic Act of 1938, as amended, and all regulations promulgated thereunder.

"**Active Pharmaceutical Ingredient**" or "**API**" means, with respect to the Finished Product, the applicable active pharmaceutical ingredients.

"**Affiliate**" means, with respect to any Person, any other Person directly or indirectly controlling or controlled by, or under direct or indirect common control with, such Person. For purposes of this definition, a Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such other Person, whether through the ownership of voting securities, by contract or otherwise.

"**[**]**" has the meaning set forth in Section 6.4.

"**[**]**" has the meaning set forth in Section 6.4.

"**Annual Net Sales**" means, with respect to any Royalty Payment Year within the Royalty Term, the aggregate sales revenues of Marathon or its Affiliates, subsidiaries or sublicensees with respect to sales of the Marathon Suspension Product in the Territory to Third Parties (excluding for such purposes any Non-Commercial Marathon Suspension Product) during such Royalty Payment Year, reduced by accruals in accordance with GAAP (to the extent applicable) for customer returns, refunds, discounts, rebates and other credits and allowances made with respect to such sales of the Marathon Suspension Product during or with respect to such Royalty Payment Year (including, but not limited to, prompt pay discounts, product returns, bad debt, Medicaid, chargebacks, fees-for-service and Tricare), which are consistent with standard industry custom and practice.

"**API Specifications**" means, with respect to the API, the applicable specifications contained in Marathon's effective FDA-approved IND for investigational product use and in Marathon's FDA-approved NDA for commercial product use, as in effect from time to time during the Manufacturing Term, which shall consider the API specifications communicated by Faes to Marathon.

"**Business Day**" means mean any day except a Saturday, Sunday or a day on which a commercial bank in Madrid, Spain, Leioa, Biscay, Spain or Chicago, Illinois, U.S.A. is authorized to close.

"**Calendar Quarter**" shall mean the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31, with the first Calendar Quarter being the one commencing closest to ten (10) calendar months prior to the then reasonably anticipated FDA Approval date for the Marathon Suspension Product in the Territory.

"**Calendar Year**" shall mean a period of twelve (12) consecutive calendar months ending on December 31.

"**Closing**" means the closing of the transactions contemplated by this Agreement.

"**Closing Payment**" means the one-time, non-reimbursable and non-creditable payment of €[**] Euros) to be made to Faes by Marathon under Section 4.3 at the Closing.

"**Cost of Goods Sold**" or "**COGS**" means, with respect to Finished Product Manufactured and supplied to Marathon by Faes under Section 6, the sum of the Direct Expenses and Manufacturing Overhead incurred by Faes in, and reasonably allocable to, the Manufacture of such Finished Product, where, as used herein:

(a) "**Direct Expenses**" means (i) those direct material and labor expenses which are incurred specifically to Manufacture such Finished Product, including costs of raw materials. Direct labor expenses include salary and fringe benefits (but exclude amounts associated with equity compensation or option plans) for personnel directly involved in the Manufacture of such Finished Product in accordance with specified quality requirements (*e.g.,* cGMP, ISO) such as production, quality control, quality assurance and other personnel who participate directly in the production of such Finished Product and components thereof, and (ii) logistics expenses for supplying such Finished Product to Marathon, including import and export duties, applicable Taxes, reasonable and customary brokerage fees, shipping insurance fees, port fees and storage fees, shipping and handling, quality control and quality assurance. Direct Expenses shall also include reasonable out-of-pocket payments to Third Parties for direct services performed in the Manufacture of such Finished Product or components thereof; and

(b) "**Manufacturing Overhead**" means a reasonable allocation of other Manufacturing expenses associated with the Manufacture of quantities of such Finished Product, including (i) Faes personnel supporting the direct Manufacturing of such Finished Product, including labor and materials for quality control, quality assurance, raw material acquisition and acceptance, document control, calibration/validation of equipment used in the Manufacture of such Finished Product and other similar expenses, (ii) depreciation of or rent/lease expenses for property, plant or equipment used in the Manufacture of such Finished Product, (iii) direct plan management (*e.g.,* supervisors and purchasing), (iv) plant services (*e.g.,* engineering and production planning) associated with the Manufacture of such Finished Product, (v) plant maintenance, (vi) costs of plant fire insurance coverage, and (vii) other direct Manufacturing costs associated with the Manufacture of such Finished Product, in each case to the extent incurred by Faes in connection with the Manufacture of such Finished Product and reasonably allocable to the Manufacture of such Finished Product.

For purposes of this Agreement, Cost of Goods Sold shall be calculated in accordance with IFRS and shall be consistent from year-to-year during the Manufacturing Term. The methodology to be used in making the allocations of any costs included in Cost of Goods Sold shall upon Marathon's request be reviewed by the Parties. As of the Effective Date, the Cost of Goods Sold with respect to a Unit manufactured and commercialized by Faes without complying with applicable U.S. FDA standards is €[**] per Unit. This Cost of Goods Sold may vary substantially if new investments or new procedures have to be implemented in the production process in order to comply with the applicable U.S. FDA standards or in case of modifications of certain specifications mutually agreed by the Parties.

"**current Good Manufacturing Practice**" or "**cGMP**" means all current good manufacturing practices (cGMP) and all applicable rules and regulations of Governmental Entities, both inside the Territory and in Spain (provided, however, that in the event any of the Manufacturing is performed in a jurisdiction or jurisdictions outside the Territory or Spain, cGMP shall also include all then applicable current good manufacturing practices and applicable rules and regulations of Governmental Entities of such additional jurisdictions), as applied at the Facility site(s) of manufacture and control, as amended from time to time and in effect during the Manufacturing Term.

"**Develop**"**,** "**Development**" and "**Developing**" means, with respect to the Marathon Suspension Product, drug development activities, including, but not limited to, CMC development, test method development and stability testing, assay development, audit development, toxicology, formulation, quality assurance/quality control development, statistical analysis, clinical studies, packaging development, regulatory affairs and the preparation, filing and prosecution of an NDA in the Territory.

"**Exclusive Manufacturing Term**" means that portion of the Manufacturing Term ending on the seventh (7^{th}) anniversary of the FDA Approval date.

"**Facility**" means Faes' manufacturing facility located in Leioa, Biscay, Spain, together with any other Faes facility in which the Manufacturing of the Finished Product occurs.

"**Faes Product**" means the deflazacort oral suspension pharmaceutical product as owned and currently supplied by Faes in certain markets in the world.

"**FDA**" means the U.S. Food and Drug Administration or any successor thereof.

"**FDA Approval**" means written approval by the FDA of the first Marathon Suspension Product NDA filed by or on behalf of Marathon or its Affiliates, subsidiaries, designees or sublicensees with an approved label indication for the treatment in humans of duchenne muscular dystrophy or another indication.

"**Finished Product**" means finished, labeled, bottled and packaged (in primary and/or secondary packaging, as mutually agreed upon by the Parties) Marathon Suspension Product for commercial sale Manufactured and supplied to Marathon by Faes under Section 6.

"**Force Majeure Event**" means, with respect to a Party, any event which is beyond the reasonable control of such Party, including, but not limited to, the following events: earthquake, storm, flood, fire or other acts of nature, epidemic, war, riot, public disturbance, strike or lockouts, customs closure, failure or default of public utilities or common carriers, government actions, terrorist attack, involuntary destruction of production facilities or the like (including, but not limited to, with respect to Faes, an inability to secure the necessary API, despite Faes' best efforts to do so, or, with respect to either or both Parties, a change in the FDA's related requirements).

"**GAAP**" means United States generally accepted accounting principles, consistently applied.

"**Governmental Entity**" means any court, agency, authority, department, legislative or regulatory body or other instrumentality of any government or country or of any national, federal state, provincial, regional, county, city or other political subdivision of any such government or any supranational organization of which any such country is a member or quasi-governmental authority or self-regulatory organization of competent authority, including, but not limited to, the FDA.

"**IFRS**" means International Financial Reporting Standards, consistently applied.

"**IND**" means an Investigational New Drug application filed with the FDA or any successor thereof in the Territory.

"**IND Materials**" means the materials listed in Appendix 1 to this Agreement.

"**Initial Manufacturing Term**" means the period commencing on the Effective Date and ending on the twentieth (20^{th}) anniversary thereof, subject to earlier termination in accordance with Section 6.12(b).

"**Initial Royalty Term**" means, with respect to the Marathon Suspension Product, the period commencing on the FDA Approval date and ending on the seventh (7^{th}) anniversary thereof.

"**Intellectual Property**" means any patents, patent applications, patent disclosures and inventions, trade secrets and other confidential and proprietary information (including, but not limited to, Inventions (whether patentable or unpatentable), and other intellectual property rights (excluding trademarks, service marks and trade names) and all copies and embodiments thereof (in whatever form or medium) and all modifications, improvements, additions, supplements, updates, renewals, continuations, continuations-in-part, reexaminations, reissues and extensions thereof.

"**Inventions**" means any inventions, developments, discoveries, improvements, works of authorship, or expressions thereof, whether or not subject to patent, copyright, trademark, trade secret protection or other intellectual property right protection (in the United States or elsewhere), and whether or not reduced to practice.

"**Know-How**" means any and all tangible and intangible information and materials, including research and development data, regulatory submissions and correspondence, manufacturing information and processes, formulations, assays, cell lines, sequences, composition of matter, constructs, discoveries, improvements, modifications, processes, methods, protocols, formulas, utility, data (including physical, chemical, biological, toxicological, pharmacological, preclinical, clinical, and veterinary data), results, inventions, know-how and trade secrets, patentable or otherwise, and all other scientific, marketing, financial and commercial information or data.

"**Knowledge**" means, with respect to a Party, the actual knowledge of such Party and its directors, managers, officers and employees, after due inquiry.

"**Law**" means any statute, law, ordinance, regulatory rule, code or order of a Governmental Entity.

"**License Term**" means the period commencing on the Effective Date and continuing in perpetuity.

"**Licensed Assets**" means the Faes Product dossier and all chemistry, manufacturing and controls **(**"**CMC**"**)** data, Intellectual Property, Know-How, Technology and other information owned or controlled (whether by license or otherwise) (a) by Faes as of the Effective Date or (b) is developed by Faes during the Manufacturing Term, which in either case would support an NDA filing in the Territory by Marathon for the Marathon Suspension Product, including, but not limited to, the IND Materials.

"**Lien**" means any lien, mortgage, security interest, pledge, defect of title and other similar encumbrance.

"**Losses**" has the meaning set forth in Section 10.10.

"**Manufacture**" and "**Manufacturing**" means all activities related to the production, manufacture, processing, filling, finishing, labeling, packaging, shipping and holding of the Finished Product or any intermediate thereof, including process development, process qualification and validation, scale-up, pre-clinical, clinical and commercial manufacture and analytical development, product characterization, stability testing, quality assurance and quality control.

"**Manufacturing Term**" means the Initial Manufacturing Term, together with any Renewed Manufacturing Terms.

"**Marathon Suspension Product**" means a deflazacort oral suspension pharmaceutical product Developed by Marathon under this Agreement based upon the Faes Product and utilizing the Licensed Assets and approved by the FDA for the treatment in humans of duchenne muscular dystrophy or another indication pursuant to an NDA filed by Marathon or its Affiliates, subsidiaries, designees or sublicensees.

"**Marathon Tablet Product**" means a deflazacort tablet pharmaceutical product for the treatment in humans of duchenne muscular dystrophy or another indication.

"**Minimum Annual Royalty Payments**" has the meaning set forth in Section 7.3(a)(ii).

"**NDA**" means a New Drug Application, validly issued and approved by the FDA or any successor thereof in the Territory.

"**NDA Approval Milestone Payment**" means the one-time, non-reimbursable and non- creditable payment of €[**] Euros) to be made to Faes by Marathon under Section 7.2 upon FDA Approval.

"**NDA Submission and Acceptance Milestone Payment**" means the one-time, non-reimbursable and non-creditable payment of €[**] Euros) to be made to Faes by Marathon under Section 7.1 upon submission to, and acceptance, by the FDA of the first Marathon Suspension Product NDA filed by Marathon or its Affiliates, subsidiaries, designees or sublicensees with a

proposed label indication for the treatment in humans of duchenne muscular dystrophy or another indication.

"**Non-Commercial Marathon Suspension Product**" means any Marathon Suspension Product Manufactured and supplied by Faes to Marathon under Section 6 and used or distributed by Marathon or its Affiliates, subsidiaries or sublicensees under or in connection with sampling programs, compassionate use/patient assistance/indigent care programs or clinical studies, programs or trials (including Marathon's Expanded Access Program).

"**Non-Commercial Marathon Suspension Product Units Prepayment Amount**" means the result of the following formula for the relevant period: [**].

"**Non-Commercial Marathon Suspension Product Units Report**" means, with respect to the Non-Commercial Marathon Suspension Product during the Royalty Term, a written report or reports showing each of the following with respect to the Non-Commercial Marathon Suspension Product in the Territory: (a) Volume in Units of Non-Commercial Marathon Suspension Product used or distributed, stating the applicable batch number of such Units; and (b) a description of the non-commercial use of those Units.

"**Non-Exclusive Manufacturing Term**" means the Manufacturing Term, excluding the Exclusive Manufacturing Term.

"**Per Unit Supply Price**" means (a) with respect to the Initial Royalty Term, €[**] Euros) per Unit; and (b) with respect to the Subsequent Royalty Term, an amount per Unit equal to Faes' actual COGS per Unit (not to exceed in any event €[**] Euros) per Unit); provided, however, that (1) in the event that Faes' actual COGS per Unit during such Subsequent Royalty Term exceed € [**] Euros) per Unit, Faes shall have the right to terminate its obligation to Manufacture and supply Finished Product to Marathon under Section 6 of this Agreement on at least twelve (12) calendar months' prior written notice of such termination (provided that, as a condition to exercising such termination right, Faes shall [**] in which case, Marathon shall be [**]; and (2) in the event that Faes' does not terminate its obligation to Manufacture and supply Finished Product to Marathon under Section 6 of this Agreement in accordance with the foregoing clause (1), [**].

"**Person**" means any individual, corporation, partnership, joint venture, limited liability company, trust or unincorporated organization or government or any agency or political subdivision thereof.

"**Pharmacovigilance Agreement**" means the mutually acceptable Pharmacovigilance Agreement to be entered into between the Parties.

"**[**]**" has the meaning set forth in Section 5.3(b).

"**Product Specifications**" means, with respect to Finished Product to be Manufactured and supplied under this Agreement, the applicable specifications contained in Marathon's effective FDA-approved IND for investigational product use and in Marathon's FDA-approved NDA for

commercial product use, as in effect from time to time during the Manufacturing Term, which shall consider the product specifications communicated by Faes to Marathon.

"**Quality Agreement**" means the quality or technical agreement covering the Finished Product to be Manufactured and supplied by Faes to Marathon under Section 6, which shall set out, among other things, the policies, procedures, and standards by which the Parties will coordinate and implement the operational and quality assurance activities and regulatory compliance objectives contemplated under this Agreement with respect to the Finished Product in and for the Territory (including, but not limited to, change control processes, changes to the Specifications and other changes to the API and/or Marathon Suspension Product).

"**Regulatory Filings**" means, with respect to the Marathon Suspension Product, any submission to the FDA of any appropriate regulatory application, and shall include any IND or NDA.

"**Renewed Manufacturing Term**" has the meaning set forth in Section 6.12(a).

"**Royalties**" has the meaning set forth in Section 7.3.

"**Royalty Term**" means the Initial Royalty Term or the Subsequent Royalty Term, as the case may be.

"**Royalty Payment Year**" means, with respect to the Marathon Suspension Product, each calendar year (or portion thereof) during the Royalty Term.

"**Royalty Payments**" has the meaning set forth in Section 7.3.

"**Sales & Royalty Report**" means, with respect to the Marathon Suspension Product during the Royalty Term, a written report or reports showing each of the following (in US Dollars) with respect to the Marathon Suspension Product in the Territory: [**].

"**[**]**" has the meaning set forth in Section 6.4.

"**Specifications**" means the API Specifications and the Product Specifications.

"**Subsequent Royalty Term**" means, with respect to the Marathon Suspension Product, the period commencing on the seventh (7^{th}) anniversary of the FDA Approval date and ending on the twentieth anniversary of the Effective Date.

"**Taxes**" means all taxes of any kind, and all charges, fees, customs, levies, duties, imposts, required deposits or other assessments, including all federal, state, local or foreign net income, capital gains, gross income, gross receipt, property, franchise, sales, VAT, use, excise, withholding, payroll, employment, social security, worker's compensation, unemployment, occupation, capital stock, ad valorem, value added, transfer, gains, windfall profits, net worth, asset, transaction, and other taxes, and any interest, penalties or additions to tax with respect thereto, imposed upon any Party by any taxing authority or other Governmental Entity under applicable Law.

"**Technology**" means any processes, techniques, batch records, specifications, formulations, assays, know-how, trade secrets and proprietary data rights.

"**Territory**" means the United States of America and its territories, possessions, commonwealths and protectorates.

"**Third Party**" means a Person who is not a Party or an Affiliate or subsidiary thereof.

"**Unit**" means one 13 ml bottle of Finished Product.

"**Unit Prepayment Amount**" means, with respect to any Calendar Quarter, the product of (a) the number of Units sold by Marathon and its Affiliates, subsidiaries and sublicensees to Third Party customers during such Calendar Quarter, times (b) the applicable Per Unit Supply Price for such Units.

1.2 Interpretation. In this Agreement, unless otherwise specified:

(a) "includes" and "including" shall mean respectively includes and including without limitation;

(b) words denoting the singular shall include the plural and vice versa and words denoting any gender shall include all genders;

(c) the Schedules and other attachments form part of the operative provision of this Agreement and references to this Agreement shall, unless the context otherwise requires, include references to the Schedules and attachments;

(d) references to Sections are to Sections of this Agreement unless otherwise specified;

(e) the headings in this Agreement are for information only and shall not be considered in the interpretation of this Agreement;

(f) any reference to "writing" or "written" includes faxes and any legible reproduction of words delivered in permanent and tangible form;

(g) the words "hereof", "herein" and "hereunder" and words of like import used in this Agreement shall refer to this Agreement as a whole and not to any particular provision of this Agreement;

(h) references to any agreement or contract are to that agreement or contract as amended, modified or supplemented from time to time in accordance with the terms hereof and thereof; and

(i) the Parties agree that the terms and conditions of this Agreement are the result of negotiations between the Parties and that this Agreement shall not be construed in favour of or against any Party by reason of the extent to which any Party participated in its preparation.

Section 2

Representations and Warranties

2.1 Representations and Warranties of Marathon. Marathon represents and warrants to Faes that:

(a) Marathon is a limited liability company duly formed under the Laws of the State of Delaware (U.S.A.);

(b) Marathon has all requisite limited liability company power and authority to execute, deliver and perform this Agreement, and, upon the execution and delivery of this Agreement by the Parties hereto, this Agreement will constitute a valid and binding obligation of Marathon, enforceable in accordance with its terms, subject to applicable bankruptcy, insolvency, fraudulent conveyance, reorganization, moratorium and other similar Laws affecting the enforcement of creditors' rights generally and to general principles of equity;

(c) the execution, delivery and performance of this Agreement by Marathon do not conflict with, or constitute a breach of, any material contract or agreement to which Marathon is a party or by which Marathon is bound;

(d) there is no litigation, proceeding or claim pending or, to Marathon's Knowledge, threatened before any Governmental Entity that would prevent the consummation of any of the transactions contemplated by this Agreement, and no consent, authorization or approval of any Third Party (including, but not limited to, a Governmental Entity) is required or necessary in connection with this Agreement or the consummation of the transactions contemplated hereby; and

(e) there is no broker, finder or financial advisor acting or who has acted on behalf of Marathon or its Affiliates who is entitled to receive any brokerage or finder's or financial advisory fee in connection with the transactions contemplated by this Agreement.

2.2 Representations and Warranties of Faes. Faes represents and warrants to Marathon that:

(a) Faes is a corporation *(sociedad anónima*) duly organized under the Laws of Spain;

(b) Faes has all requisite corporate power and authority to execute, deliver and perform this Agreement, and, upon the execution and delivery of this Agreement by the Parties hereto, this Agreement will constitute a valid and binding obligation of Faes, enforceable in accordance with its terms, subject to applicable bankruptcy, insolvency, fraudulent conveyance, reorganization, moratorium and other similar Laws affecting the enforcement of creditors' rights generally and to general principles of equity;

(c) the execution, delivery and performance of this Agreement by Faes do not conflict with, or constitute a breach of, any material contract or agreement to which Faes is a party or by which Faes is bound;

(d) there is no litigation, proceeding or claim pending or, to Faes' Knowledge, threatened before any Governmental Entity (i) relating to the Faes Product or any of the Licensed Assets, or (ii) that would prevent the consummation of any of the transactions contemplated by this Agreement,

and no consent, authorization or approval of any Third Party (including, but not limited to, a Governmental Entity) is required or necessary in connection with this Agreement or the consummation of the transactions contemplated hereby;

(e) Faes has, and at the Closing will have, (i) good and valid title to the Faes Product and the Licensed Assets to be licensed to Marathon by Faes under this Agreement, with the full right, power and authority to grant to Marathon the licenses contemplated by this Agreement, and (ii) a valid, irrevocable and perpetual license and right to hold, use and sublicense to Marathon the Licensed Assets to be sublicensed to Marathon by Faes under this Agreement, in each case free and clear of any and all Liens;

(f) neither Faes nor its Affiliates have granted to any Third Party any license, sublicense or other right or interest in or with respect to the Faes Product or any of the Licensed Assets in or with respect to the Territory, and no Third Party has a superior right to Faes or its Affiliates in or with respect to the Faes Product or the use of any of the Licensed Assets in or with respect to the Territory;

(g) to Faes' Knowledge, no Third Party is engaging in any activity that contravenes, infringes or encroaches upon, misappropriates or otherwise violates any of the Licensed Assets. None of the Licensed Assets (i) contravenes, infringes or encroaches upon, misappropriates or otherwise violates the intellectual property or other proprietary rights or interests of any Third Party or (ii) is involved in any cancellation, nullification, reissue, interference, re-examination, or opposition proceedings, and no inequitable conduct that would be in violation of 37 C.F.R. § 1.56, or its foreign equivalent, if applicable, has been committed in the prosecution of any of the same; all maintenance fees, annuity fees, renewal fees and similar payment obligations with respect to the Licensed Assets have been timely paid; no litigation, proceeding or claim is pending or, to Faes' Knowledge, threatened against Faes or its Affiliates (A) based upon, challenging or seeking to deny or restrict the use of any of the Licensed Assets or (B) alleging that the use of any of the Licensed Assets contravenes, infringes or encroaches upon, misappropriates or otherwise violates the intellectual property or other proprietary rights or interests of any Third Party; and

(h) there is no broker, finder or financial advisor acting or who has acted on behalf of Faes or its Affiliates who is entitled to receive any brokerage or finder's or financial advisory fee in connection with the transactions contemplated by this Agreement.

Section 3

License Grant

3.1 License Grants. During the License Term, Faes hereby grants to Marathon (a) a right and license in, to and under the Faes Product and (b) a right and license or sublicense, as the case may be, in, to and under the Licensed Assets, in each case (i) including the right to grant sublicenses or sub-sublicenses, as the case may be, with respect thereto through multiple tiers to Marathon's Affiliates or subsidiaries and/or Third Parties (on written notice to Faes), and (ii) to research, Develop, seek and obtain regulatory approval for, market, promote, distribute, offer to

sell and/or sell, use, commercialize and, solely as expressly permitted under this Agreement, manufacture (or have manufactured by an Affiliate, subsidiary or Third Party) the Marathon Suspension Product in and for the Territory. The foregoing rights and licenses shall be exclusive (even as to Faes and its Affiliates) during the Manufacturing Term and non-exclusive thereafter. If Marathon grants a sublicense or sub-sublicense, as the case may be, with respect to its rights and licenses under this Section 3 to a Third Party, (A) each such sublicense or sub-sublicense shall be in writing, (B) the terms and conditions of such sublicense or sub-sublicense shall be consistent with the terms and conditions of this Agreement and shall not jeopardize, reduce or in any other way limit Faes' rights under this Agreement and (C) Marathon shall be liable for any such sublicensee's or sub-sublicensee's breach of this Agreement or Losses caused to Faes as a result of such sublicenses or sub-sublicenses.

3.2 Right to List or Otherwise Include Licensed Assets in Regulatory Filings. The rights and licenses granted in this Section 3 include the right for Marathon or its Affiliates, subsidiaries, designees or sublicensees to list or otherwise include, as appropriate, any Licensed Assets in any Regulatory Filings (including the "Orange Book", if applicable) with respect to the Marathon Suspension Product in the Territory.

3.3 Reservation of Rights by Faes and its Affiliates.

(a) The Parties acknowledge and agree that, subject to the terms and conditions of this Agreement, Faes and its Affiliates reserve all rights under the Licensed Assets to research, develop and manufacture the Faes Product for any and all purposes both inside the Territory (with Marathon's prior written consent, not to be unreasonably withheld) or outside the Territory.

(b) The rights and licenses granted to Marathon under this Agreement shall not include any right to research, develop, seek and obtain regulatory approval for, market, promote, distribute, offer to sell and/or sell, market, use, distribute, commercialize, import, manufacture and/or have manufactured the Faes Product and/or the Marathon Suspension Product outside the Territory, provided, however, that, notwithstanding the foregoing, Marathon may research, develop, import and (to the extent expressly permitted under this Agreement) manufacture and/or have manufactured Marathon Suspension Product outside the Territory solely for purposes of seeking and obtaining FDA Approval for and marketing, promoting, distributing, offering to sell and/or selling, marketing, using, distributing or commercializing the Marathon Suspension Product in the Territory.

(c) Notwithstanding the rights and licenses granted to Marathon under this Agreement or anything else in this Agreement to the contrary, for the avoidance of doubt, (i) Faes shall retain the right (which shall be exclusive vis-a-vis Marathon and its Affiliates and sublicensees) under the Faes Intellectual Property and Know-How to research, develop, make and have made, use, market, distribute, offer for sale, sell and import the Faes Product outside the Territory for any and all purposes, including seeking to obtain regulatory approval for the Faes Product in all countries outside the Territory; (ii) Faes shall retain the right (which shall be exclusive vis-à-vis Marathon and its Affiliates and sublicensees) to research, develop, make, have made, use, market, offer for sale, sell and import the Faes Product for any and all uses in all countries, territories or jurisdictions

other than the Territory; and (iii) Faes shall retain the right (which shall be exclusive vis-à-vis Marathon and its Affiliates and sublicensees) with respect to the Faes Product to obtain, register or file any right and interest outside the Territory in and to all issued patents or pending patent applications or similar rights, including all provisional patent applications, substitutions, continuations, continuations-in-part, divisions and renewals, all letters patent granted thereon, and all patents-of-addition, reissues, re-examinations and extensions or restorations by existing or future extension or restoration mechanisms (including regulatory extensions), and all supplementary protection certificates.

(d) For purposes of rights and licenses granted to Marathon under this Agreement, for the avoidance of doubt, Faes expressly declares, and Marathon expressly acknowledges, that as of the Effective Date the Licensed Assets do not include any patents or pending patent applications in the Territory with respect to, or that claim, the Faes Product or the Licensed Assets, and Faes shall have no liability or obligation whatsoever with respect to pursuing, obtaining, registering or filing patents and/or patent applications in the Territory with respect to, or that claim, the Faes Product or the Licensed Assets. This Agreement does not prohibit Marathon, to the extent Marathon deems it appropriate, to pursue, obtain, register or file patents and/or patent applications in the Territory in the name of Marathon or its Affiliates, subsidiaries or sublicensees with respect to the Marathon Suspension Product.

3.4 No Implied License. Only the rights and licenses expressly granted to Marathon under this Agreement shall be of legal force and effect as between the Parties, and no other right or license shall be created or deemed granted by or under this Agreement, whether by implication, estoppel or otherwise.

Section 4

Closing

4.1 Closing. The Closing shall occur in person or by electronic means at Marathon's offices on May 12, 2015 at 10:00 a.m. Central Standard Time.

4.2 Faes Closing Deliveries. Within three (3) calendar days after the Effective Date, Faes shall deliver to Marathon all of the IND Materials. No later than February 15, 2016, Faes shall deliver to Marathon all of the other Licensed Assets.

4.3 Marathon Closing Deliveries. At the Closing, Marathon shall deliver to Faes the Closing Payment by wire transfer of cash or other immediately available funds to the account designated by Faes by written notice to Marathon at least two (2) Business Days prior to the Closing.

Section 5

Development

5.1 Development Responsibility and Authority.

(a) Marathon shall have responsibility and authority (with Faes' input, advice and assistance) with respect to conducting, in Marathon's discretion and at Marathon's expense (except

as otherwise set forth in this Agreement), such research and preclinical, clinical, regulatory and other Development of the Marathon Suspension Product for the treatment in humans of duchenne muscular dystrophy or another indication designated by Marathon, in its discretion, in and for the Territory. Marathon shall attempt to coordinate such Development activity with Marathon's Development activity with respect to the Marathon Tablet Product, with the goal being to have the NDAs for both the Marathon Suspension Product and the Marathon Tablet Product approved by the FDA at approximately the same time. Without limiting the generality of the foregoing, Marathon shall have responsibility and authority (in Marathon's discretion and at Marathon's expense) for and with respect to (a) determining the regulatory plans, strategies and Regulatory Filings for the Marathon Suspension Product in the Territory, (b) making all Regulatory Filings (either itself or through its Affiliates, subsidiaries, designees or sublicensees) with respect to the Marathon Suspension Product in the Territory in the name of Marathon or its Affiliates, subsidiaries, designees or sublicensees and (c) obtaining, owning and/or maintaining the NDA(s) and other Regulatory Filings with respect to the Marathon Suspension Product in the Territory in the name of Marathon or its Affiliates, subsidiaries, designees or sublicensees.

(b) If Marathon does not obtain FDA Approval on or prior to December 31, 2019, Marathon may terminate this Agreement in its entirety on thirty (30) calendar days' written notice to Faes. In such event, as well as in the event that Faes terminates this Agreement under and in accordance with Section 10.12 due to Marathon's material and uncured breach of this Agreement, (i) any and all rights granted to Marathon or its Affiliates, subsidiaries or sublicensees under this Agreement (including the rights and licenses granted to Marathon under Section 3 and any sublicenses or sub-sublicenses granted by Marathon or its Affiliates or subsidiaries hereunder) shall terminate on the effective date of such applicable termination, (ii) Marathon and its Affiliates, subsidiaries and sublicensees shall immediately cease using any Faes Technology, Intellectual Property or Know-How included in the Licensed Assets and any Technology, Intellectual Property or Know-How developed by Marathon, its Affiliates, subsidiaries and sub-licensees using any Faes Technology, Intellectual Property or Know-How included in the Licensed Assets, (iii) Faes shall retain, and not return to Marathon, any payments paid to Faes by Marathon, and Marathon shall pay to Faes any payment due under this Agreement prior to the effective date of such applicable termination and (iv) Marathon, its Affiliates, subsidiaries and sub-licensees shall destroy or delete (including from all computers and technological devices, as far as possible, except for back-ups or other security filing systems if such deletion is not possible, but being in this case Marathon obliged not to access), and cause its Affiliates, subsidiaries and sublicensees to destroy or delete (including from all computers and technological devices, as far as possible, except for back-ups or other security filing systems if such deletion is not possible, but being in this case Marathon obliged not to access) all confidentiality and proprietary information included within the Licensed Assets (including Faes' Technology, Intellectual Property and Know-how), without keeping copies, summaries, excerpts or samples thereof. In the event of the breach or threatened breach of this Section 5.1(b) by Marathon, Faes shall be entitled to request, before the arbitration court or otherwise, all available legal remedies with respect thereto, including seeking indemnification (the limitations set forth in the first proviso of Section 10.11 not being applicable in this particular case) or seeking specific performance or

other equitable remedies in order to cause Marathon to immediate cease such breach(es) or threatened breach(es) or to otherwise comply with this Section 5.1(b).

5.2 Parties' Collaboration Regarding Manufacturing Research and Development. Without prejudice to Section 5.1, (a) the Parties shall in good faith work collaboratively with each other with respect to the manufacturing research and development of the Marathon Suspension Product for the Territory as necessary to support Marathon's filing of the Marathon Suspension Product NDA(s) in the Territory, including, but not limited to, negotiating, mutually agreeing upon and implementing manufacturing research and developments with respect to the Marathon Suspension Product which are convenient, advisable or appropriate in order to make the Marathon Suspension Product more competitive and commercially viable in the Territory (including, but not limited to, addressing child-resistant cap/closure and stability issues with respect to the Territory and, at a later date after filing of the Marathon Suspension Product NDAs in the Territory, addressing product taste issues), with the financial terms of such manufacturing research and development to be part of such negotiations, and (b) Faes shall use reasonable efforts with respect to such manufacturing research and development of the Marathon Suspension Product for the Territory (including the implementation thereof) as necessary to support a Marathon Suspension Product NDA filing in the Territory by Marathon, including, but not limited to, using its best efforts, within Faes' control, in order to assist Marathon (subject to Section 5.3), wherever reasonably possible, to meet Marathon's launch timing of the Marathon Suspension Product in the Territory (which is currently foreseen for [**]) so that it is coincident with the timing of Marathon's launch of the of the Marathon Tablet Product in the Territory. The direct costs incurred by Faes in connection with such collaboration, research and development shall be borne by Marathon, subject to mutual agreement of the Parties pursuant to the financial terms negotiations provided for above and Section 5.3(a).

5.3 Development Costs and Expenses. With respect to the foregoing Development activities with respect to the Marathon Suspension Product in and for the Territory, except as otherwise set forth in this Agreement and in any event without duplication:

(a) Marathon shall fund all Development costs and expenses associated with gaining FDA Approval of the Marathon Suspension Product NDA in the Territory, up to an amount of US$[**] Dollars). Once this amount has been reached, Marathon shall not be obligated to continue funding the Development for such purposes. [**].

(b) Within [**] calendar days after the Effective Date, Faes will [**] and [**] and will [**].

(c) Marathon shall fund (subject to Marathon's prior written approval) the CMC development work costs necessary to provide for a compliant NDA dossier for the Marathon Suspension Product for the Territory.

(d) Marathon shall pay to Faes a reasonable, mutually agreed upon hourly fee (based upon the hourly rates listed in Appendix 2 to this Agreement) for [**] and [**] with respect to the [**] under this Agreement. [**] will be [**].

(e) Faes shall not be obligated to fund any Development costs or expenses associated with gaining FDA Approval of the Marathon Suspension Product in the Territory (including, but not limited to, any costs and expenses associated with complying with a U.S. FDA Pre Approval Inspection of the Facility), unless such costs and expenses are financed by Marathon or are otherwise agreed to by Faes.

(f) Each of the Parties agree that in performing its obligations under this Agreement (including, but not limited to, this Section 5): (a) it shall comply with all applicable Laws, including, but not limited to, all applicable regulatory standards, including cGMP; and (b) it will not employ or use any Person that has been debarred by the FDA under Section 306(a) or 306(b) of the Act.

Section 6

Finished Product Supply

6.1 Supply of Finished Product. Subject to the terms and conditions of this Agreement, during the Manufacturing Term, Faes shall Manufacture and supply the Finished Product to Marathon, and Marathon shall acquire the Finished Product from Faes, in and for the Territory as follows:

(a) on an exclusive basis during the Exclusive Manufacturing Term; and

(b) on a non-exclusive basis during the Non-Exclusive Manufacturing Term.

Faes acknowledges and agrees that the Finished Product Manufactured and supplied to Marathon during the Manufacturing Term shall constitute the only deflazacort oral suspension product manufactured or supplied by Faes or its Affiliates for use in the Territory during the Manufacturing Term.

Marathon acknowledges that (except as expressly provided for under this Agreement) Marathon shall purchase exclusively from Faes all of its requirements for Marathon Suspension Product for the Territory during the Exclusive Manufacturing Term, and that (except as expressly provided for under this Agreement) Marathon shall cause Marathon's Affiliates, subsidiaries and sub-licensees to purchase exclusively from Faes all of its requirements for Marathon Suspension Product for the Territory during the Exclusive Manufacturing Term.

6.2 Forecasts and Purchase Orders.

(a) On or before the first [**] calendars days of each Calendar Month during the Manufacturing Term, Marathon shall provide to Faes a written, good faith rolling forecast (each, a "**Regular Forecast**") of the quantity of Finished Product that Marathon estimates ordering in or with respect to the coming [**] calendar month period of time under this Agreement. Only the first [**] calendar months of each Regular Forecast shall be binding with regard to the estimated

quantities of Finished Product specified therein; the remaining [**] calendar months of each Regular Forecast shall be non-binding with regard to the estimated quantities of Finished Product specified therein.

However, [**] months before the anticipated first requested delivery date of Finished Product under this Section 6, Marathon shall provide to Faes with a non-binding forecast of the quantity of Finished Product Marathon estimates ordering in or with respect to the coming [**] calendar months commencing with the month of the anticipated first requested delivery date (the "**First Forecast**").

(b) Firm purchase orders (each, a "**Purchase Order**") for quantities of Finished Product to be manufactured and supplied by Faes under this Agreement shall be submitted to Faes by Marathon on or before the [**] during the Manufacturing Term. Such Purchase Orders shall, among other things, specify the desired delivery date of the applicable Finished Product, which specified desired delivery date shall not, in any event, be less than [**] calendar months after Marathon's delivery of such Purchase Order to Faes.

6.3 Supply, Acceptance, Delivery and Remedies.

(a) During the Manufacturing Term, Faes shall supply the quantity of Finished Product specified in each Purchase Order on the specified desired delivery date; provided that (i) such Purchase Order has been submitted to Faes at least [**] calendar months prior to the specified desired delivery date in accordance with this Section 6 and (ii) no Force Majeure Event is preventing Faes from timely performing such supply obligation.

(b) Prior to the shipment of any Finished Product to be Manufactured and supplied by Faes to Marathon under this Agreement, Faes shall deliver to Marathon, for Marathon's review and approval, the following release documentation with respect to such shipment of Finished Product: the applicable certificate of analysis, certificate of compliance, executed batch records, test records and other release documents specified in the Quality Agreement (collectively, the "**Release Documentation**"). Marathon shall have [**] Business Days after its receipt of such Release Documentation to review such Release Documentation in order to determine, based upon such Release Documentation, whether such Finished Product does or does not conform to the applicable Specifications, cGMPs and Laws, the applicable Purchase Order and this Agreement and to either accept or reject such shipment of Finished Product as conforming or non-conforming by delivering written notice thereof to Faes prior to the end of such [**] Business Day period. If Marathon either accepts such shipment of Finished Product as conforming, or fails to reject such shipment of Finished Product as non-conforming, in either case in writing in accordance with the preceding sentence, Faes shall be permitted to ship such Finished Product to Marathon, with (i) delivery of such Finished Product to be made CIF sea or CIP air (at Faes' choice) (Incoterms 2000) from Bilbao, Spain port to Marathon's specified designation using a freight carrier chosen by Faes and reasonably acceptable to Marathon and (ii) title and risk of loss therein and thereto passing to Marathon upon delivery of such Finished Product to the applicable freight carrier at Bilbao, Spain port.

(c) Upon Marathon's receipt of any such shipment of Finished Product, Marathon shall perform a visual inspection thereof to determine whether such Finished Product does or does not conform to the applicable Specifications, cGMPs and Laws, the applicable Purchase Order and this Agreement. Marathon shall notify Faes in writing without unreasonable delay if it determines based on such inspection that such shipment of Finished Product is non-conforming in any respect. Except as to defects that could not reasonably have been discovered by such visual inspection, the Finished Product in such shipment shall be deemed to have been accepted by Marathon if Faes has not received such written notice of non-conformance from Marathon within [**] calendar days after the date of Marathon's receipt of such shipment. Marathon shall notify Faes of any latent defects in such shipment of Finished Product that could not reasonably have been discovered by such visual inspection within [**] calendar days after discovery thereof.

(d) If Faes receives timely notice from Marathon pursuant to this Section 6.3 of the non-conformity of a shipment of Finished Product, and agrees that such shipment is non-conforming (or, in the event that Faes disagrees that such shipment is non-conforming, if an independent laboratory or expert mutually acceptable to the Parties determines that the shipment is non-conforming), Faes shall, at Marathon's election, [**].

6.4 Failure to Supply Finished Product. In the event that (A) Faes becomes aware at any time during the Manufacturing Term that it is unable or likely to be unable to fulfill any Marathon Purchase Order in a timely manner (whether as a result of a Force Majeure Event or otherwise), or (B) Faes materially breaches its main obligations under Section 6 of this Agreement, Faes shall immediately notify Marathon thereof (which notification shall include the underlying reason for such supply delay or breach, the proposed remedial measures and the date that such supply delay is expected to end or such material breach is expected to be cured). In the event that Faes so notifies Marathon of [**] (each, a "**Supply Failure**"), the Parties shall [**]. In the event that the Parties [**], and with respect to [**] in accordance with Section [**], (a) Marathon shall [**], (b) if [**], (c) Marathon shall [**] and (d) Marathon shall [**]. Faes shall [**], including, but not limited to, [**] with Marathon [**]. Without prejudice of the foregoing, if Faes [**], Faes shall [**].

6.5 Supply Price; Invoicing.

(a) In consideration for each Unit of Finished Product Manufactured and supplied by Faes to Marathon under this Section 6, Marathon shall pay to Faes the Per Unit Supply Price, which, the Parties expressly acknowledge and agree, with respect to the Initial Royalty Term, constitutes a prepayment, in part, of the Royalty Payments otherwise payable to Faes by Marathon under Section 7.3 with respect to such Initial Royalty Term.

(b) With respect to each shipment of Finished Product Manufactured and supplied by Faes to Marathon under this Section 6, Faes shall invoice Marathon for an amount equal to the product of (a) the number of Units included in such shipment of Finished Product, times (b) the Per Unit Supply Price, promptly after delivery of such shipment of Finished Product to Marathon. Marathon shall pay Faes such invoiced amount within [**] calendar days after its receipt of the invoice for such shipment of Finished Product.

6.6 Finished Product Warranty. Faes represents, warrants and covenants to Marathon that: (a) the Finished Product to be Manufactured and supplied by Faes to Marathon under this Section 6 shall conform to the applicable Specifications, cGMPs, Laws and the Quality Agreement; (b) Faes will convey good and valid title to the Finished Product Manufactured and supplied by Faes to Marathon under this Section 6, free and clear from any and all Liens; (c) as of the time of delivery of any such Finished Product to Marathon hereunder, such Finished Product will not be adulterated or misbranded under the Act or other applicable Law; and (d) as of the time of delivery of any such Finished Product to Marathon hereunder, such Finished Product will have a remaining shelf equal to or greater than [**] percent ([**]%) of its then current FDA approved shelf life (and, in no event, less than [**] months of its remaining shelf-life).

6.7 [**]. During the Exclusive Manufacturing Term, Marathon will [**]; provided, however, that (i) those [**] will be [**] under this Agreement, [**] under this Agreement; and (ii) [**] under this Section 6.7 shall be [**].

6.8 API, Components and Raw Materials. Faes shall be responsible, at its cost, for the procurement, manufacture and qualification of the API and any components or raw materials required for the Manufacture of the Finished Product.

6.9 Manufacturing Records. Faes shall maintain and/or cause its Third Party suppliers or API, components and raw materials to maintain all records and other materials necessary to comply with applicable cGMPs and all applicable Laws relating to the Manufacture and supply of the Finished Product under this Section 6. All such materials shall be maintained for such period as may be required by applicable Law; provided, however, that all records relating to the Manufacturing (including stability and quality control) of each batch of Finished Product shall be retained until at least the [**] of the end of its then-current FDA approved shelf-life, unless a longer period is required by applicable Law. Notwithstanding anything in this Section 6.9 to the contrary, if Faes desires to destroy or discard any such materials, Faes shall notify Marathon (with specificity as to which materials that it desires to destroy or discard) in writing prior to doing so, and Marathon shall have the right to take custody of such materials within [**] Business Days after receipt of such notice.

6.10 Audits and Facility Access. During the Manufacturing Term, Faes shall allow, during regular business hours and on reasonable prior notice, Marathon's quality assurance, quality control, compliance and other relevant personnel (including Marathon's consultants provided they are under the same confidentiality obligations as Marathon regarding Faes confidential information), to audit the Facilities and related documentation and the Manufacture of Finished Product to be Manufactured and supplied under this Section 6 [**] without cause and additional times per Calendar Year as necessary for cause (each, an "**Audit**"). The purpose of any such Audit shall solely be to assess compliance with applicable cGMPs and Laws. Furthermore, Faes will allow inspectors from the FDA and other relevant Governmental Entities in the Territory to perform required inspections of such Facilities and related documentation with respect to the Finished Product or the API. Faes shall, without delay, inform Marathon of any such proposed or unannounced FDA or other such

Governmental Entity inspections of such Facilities. Faes agrees to permit one or more qualified representative(s) of Marathon to be present on site during any such FDA or other such Governmental Entity inspections pertaining to the Finished Product or the API. Faes shall, without undue delay, provide a summary report of the results of any such FDA or other such Governmental Entity inspection to Marathon. Faes shall, without delay, notify Marathon of any FDA or other such Governmental Entity request for samples of the Finished Product or the API, as applicable.

6.11 Quality Agreement and Pharmacovigilance Agreement.

(a) Within [**] Business Days after the Effective Date, the Parties shall do their best efforts to enter into the Quality Agreement. Marathon shall use its best efforts to prepare and circulate the first version of this Quality Agreement (in a reasonable and customary format) within [**] Business Days after the Effective Date.

(b) The Parties agree that Marathon shall have primary responsibility for the monitoring of all clinical experiences and filing of all required reports concerning the Marathon Suspension Product in the Territory throughout its development and commercialization in the Territory. Faes (either itself or through a clinical research organization with whom it has contracted), its Affiliates or its Third Party partners shall have primary responsibility for the monitoring of all clinical experiences and filing of all required reports concerning the Faes Product in the countries, territories or jurisdictions in which Faes (either directly or indirectly) or its Affiliates or Third Party partners commercialize, or conduct clinical or other activity with respect to, the Faes Product. The Parties shall cooperate to develop methods and/or procedures for sharing information relating to such clinical experiences in accordance with safety reporting requirements of the respective applicable Governmental Entities and all applicable Laws. Specific details regarding the management of information adverse events related to the clinical development and use of the Marathon Suspension Product in the Territory will be delineated in the Pharmacovigilance Agreement to be entered into by the Parties within [**] Business Days after the Effective Date, provided, however, that in any event each Party agrees to provide the other Party with such information regarding adverse events with respect to the Marathon Suspension Product or the Faes Product, as the case may be, within such time frames as are required by applicable Laws.

6.12 Manufacturing Term.

(a) Upon expiry of the Initial Manufacturing Term or a Renewed Manufacturing Term, as the case may be, the Manufacturing Term shall automatically be renewed for a ten (10) calendar year period (each, a "**Renewed Manufacturing Term**"), unless either Party gives the other Party written notice of non-renewal at least twelve (12) calendar months prior to the expiry of such Initial Manufacturing Term or Renewed Manufacturing Term, as the case may be.

(b) The Manufacturing Term may be terminated at any time by mutual written agreement of the Parties.

Section 7

Other Financial Provisions

7.1 NDA Submission and Acceptance Milestone Payment. Upon submission to, and acceptance, by the FDA of the first Marathon Suspension Product NDA filed by Marathon or its Affiliates, subsidiaries, designees or sublicensees with a proposed label indication for the treatment in humans of duchenne muscular dystrophy or another indication, Marathon shall pay to Faes the NDA Submission and Acceptance Milestone Payment, due and payable within [**] Business Days thereafter by wire transfer of cash or other immediately available funds to the account designated in writing by Faes.

7.2 NDA Approval Milestone Payment. Upon FDA Approval, Marathon shall pay to Faes the NDA Approval Milestone Payment, due and payable within [**] Business Days thereafter by wire transfer of cash or other immediately available funds to the account designated in writing by Faes, if (a) [**], Faes shall [**] or (b) Marathon shall [**]. Notwithstanding the foregoing, if Faes [**], Marathon shall [**] in accordance with Section 6.

7.3 Royalty Payments. In consideration for the rights and licenses granted to Marathon under Section 3 of this Agreement, Marathon shall pay royalties to Faes as set forth in this Section 7.3 (collectively, the "**Royalties**"), with the amounts payable under this Section 7.3 sometimes being collectively referred to in this Agreement as the "**Royalty Payments**"):

(a) Initial Royalty Term. With respect to the Initial Royalty Term:

(i) Marathon shall pay to Faes Royalties (which the Parties expressly acknowledge and agree are inclusive of the supply price prepaid to Faes by Marathon under Section 6 with respect to Finished Product Manufactured and supplied by Faes to Marathon thereunder) which shall be calculated as a percentage of - or as a fixed payment with respect to - the Annual Net Sales of the Marathon Suspension Product in the Territory by Marathon and its Affiliates, subsidiaries or sublicensees per Calendar Quarter during the Initial Royalty Term in accordance with the table below (with each Royalty percentage or fixed payment, as the case may be, set forth below applicable only with respect to Annual Net Sales of Marathon Suspension Product within the applicable range set forth below):

Annual Net Sales	***Royalty Percentage / Fixed Payment***
[**]	[**]
[**]	[**]
[**]	[**]

In making any Royalty Payments with respect to Royalties under this Section 7.3(a), Marathon shall [**].

(ii) In no event shall the aggregate Royalties (which, for such purposes, shall [**] in making the applicable Royalty Payment in accordance with Section 7.3(a)(i)) payable to Faes by Marathon under this Section 7.3(a) with respect to a first seven (7) Calendar Years ending

during the Initial Royalty Term be less than the following (the "**Minimum Annual Royalty Payments**"):

Year Post Launch	*Minimum Annual Royalty Payment*
[**]	€0.5 million
[**]	[**]
[**]	€1.5 million

(b) Subsequent Royalty Term. With respect to the Subsequent Royalty Term, Marathon shall pay to Faes Royalties equal to [**] percent ([**]%) of the Annual Net Sales of the Marathon Suspension Product in the Territory by Marathon and its Affiliates, subsidiaries, sublicensees or sub-sublicensees per Calendar Quarter.

(c) Sales & Royalty Reports. Within [**] calendar days after each Calendar Quarter during the Royalty Term, Marathon shall provide to Faes a Sales & Royalty Report for such Calendar Quarter.

(d) Non-Commercial Marathon Suspension Product Units Reports. Within [**] calendar days after each Calendar Quarter during the Royalty Term, Marathon shall provide to Faes a Non-Commercial Marathon Suspension Product Units Report for such Calendar Quarter.

(e) Royalty Payments. Royalty Payments payable under this Section 7.3 shall be calculated and paid on a Calendar Quarter basis by wire transfer of cash or other immediately available funds to the account designated in writing by Faes, within [**] calendar days after the end of each Calendar Quarter during the Royalty Term.

7.4 Payment Terms; Currency. All payments to a Party by the other Party under this Agreement shall be made by wire transfer of cash or other immediately available funds to the credit of such bank account of such Party as may be designated by such Party in this Agreement or on written notice to the other Party from time to time as provided for in this Agreement. Any payment under this Agreement which falls due on a date which is not a Business Day shall be made on the next succeeding Business Day. Except as expressly set forth in this Agreement, all payments under this Agreement shall be made in US Dollars.

7.5 Taxes; Withholding. Each Party shall bear sole responsibility with respect to any Taxes payable with respect to payments or other amounts received by such Party under this Agreement. To the extent that a Party making payments to the other Party under this Agreement is required under applicable Law to deduct and withhold an amount from such payment(s), such Party shall entitled to do so and such withheld amount(s) shall be treated for all purposes of this Agreement as having been paid, and proof of payment from the applicable taxing authority shall be provided to the Party on whose behalf the applicable Tax was paid.

7.6 Records and Audits.

(a) Marathon shall keep complete, true and accurate books and records in accordance with GAAP in relation to this Agreement and the transactions contemplated hereby, including, Annual Net Sales, Royalties and Royalty Payments. Faes shall keep complete, true and accurate books and records in accordance with IFRS in relation to this Agreement and the transactions contemplated hereby, including COGS. Each Party will keep such books and records for at least [**] calendar months following the applicable Calendar Quarter to which they pertain.

(b) Not more often than [**] during the Royalty Term, Faes shall have the right for a period of [**] calendar months following receipt of the applicable Sales & Royalty Report and the Non-Commercial Marathon Suspension Product Units Report to audit, whether by itself or through its Affiliate(s) and/or to appoint an internationally-recognized independent accounting firm approved by Marathon (whether Faes, its Affiliate or an independent accounting firm, the "**Auditor**") to audit the relevant books and records of Marathon solely with respect to such Sales & Royalty Report and Non-Commercial Marathon Suspension Product Units Report for purposes of verifying the accuracy thereof and of the Annual Net Sales, Royalties and Royalty Payments set forth therein. Where the Auditor is not Faes, such Auditor shall execute and deliver to Marathon a confidentiality agreement, in form and substance acceptable to Marathon, have the right to disclose to Faes and/or other Affiliates of Faes its conclusions regarding the applicable Sales & Royalty Report, Non-Commercial Marathon Suspension Product Units Report and the Annual Net Sales, Royalties and Royalty Payments set forth therein. Faes agrees to hold in confidence all information received and all information learned in the course of any such audit, whether received or learned directly or through an Affiliate or other Auditor), except to the extent that such information is not confidential and/or it is necessary to disclose it to enforce its rights under this Agreement or if disclosure is required by applicable Law.

(c) [**].

(d) If there is a dispute between the Parties following any audit performed pursuant to this Section 7.6 which is not resolved by mutual agreement of the Parties, either Party may [**]. In the event an [**], the Parties shall [**]: (i) the Party [**] of this Section 7.6(d); (ii) within [**] Business Days after the [**], the Parties shall [**]; (iii) the [**]; (iv) the [**]; (v) the [**] of any of the terms and conditions thereof; and (vi) [**].

7.7 Right of Setoff. The Parties hereby expressly acknowledge and agree that each Party shall have the right to offset against any undisputed payments payable to the other Party under this Agreement any amounts owed by such other Party under this Agreement.

Section 8

Infringement of Licensed Assets by Third Parties

8.1 Infringement. Each Party shall promptly notify the other Party of any actual, suspected or threatened infringement, violation or misappropriation of the Licensed Assets within the Territory ("**Infringement**") that comes to its attention.

8.2 Right to Bring Action. Marathon shall have the sole right (either itself or through its Affiliates, designees or sublicensees) to send notices and bring and conduct actions in relation to any Infringement. Faes will co-operate fully with Marathon or its Affiliates, designees or sublicensees, as the case may be, in taking all reasonable steps requested thereby in connection with any Infringement action, including joining in legal proceedings. Marathon shall bear the out- of pocket costs of any such legal proceedings, and shall be entitled to [**] percent ([**]%) of any damages, account of profits and/or awards of costs recovered.

8.3 Exception. In the event that Marathon does not take reasonable steps to prevent any individual Infringement within [**] days of becoming aware or receiving notice thereof, Faes shall thereafter have the right (but shall not be under any obligation in this regard) to send notices and bring and conduct actions in relation to such Infringement. Marathon will co-operate fully with Faes in taking all reasonable steps requested by Faes in connection with any such Infringement action, including joining in legal proceedings. Faes shall bear the costs of any such legal proceedings, and shall be entitled to [**] percent ([**]%) of any damages, account of profits and/or awards of costs recovered.

8.4 Settlements. The Parties shall reasonably consult with each other with respect to any such Infringement before accepting any settlement thereof or any judicial finding which is reviewable by a higher authority with respect thereto.

Section 9

Perpetuity

At the expiry of the Royalty Term, the rights and licenses granted to Marathon under Section 3 shall automatically convert into royalty-free, fully paid-up and non-assessable rights and licenses.

Section 10

Miscellaneous

10.1 Governing Law. This Agreement shall be governed by, and construed under, the laws of the Kingdom of Spain.

10.2 Assignment. Neither Party may assign its rights and obligations under this Agreement without the other Party's prior written consent, except that (a) either Party may assign its rights and obligations under this Agreement or any part hereof to one or more of its Affiliates without the consent of the other Party; and (b) Marathon may assign this entire Agreement without Faes' prior written consent to a Third Party acquirer, successor or designee (i) to all or substantially all of Marathon's business or assets or (ii) to all of Marathon's rights with respect to the Marathon Suspension Product in the Territory, provided, however, that the assigning Party shall remain responsible for the assignee's full and accurate performance of its pre and post-assignment obligations under this Agreement. Any attempted assignment in contravention of the foregoing shall be void.

Further, and without limiting the foregoing. Marathon and Faes shall be permitted to engage their respective Affiliates and/or subsidiaries to perform services to assist the respective Party in performing its respective obligations under this Agreement, including with respect to the Manufacturing, Development and/or commercialization of the Marathon Suspension Product in and for the Territory, provided that the applicable Party shall remain liable for the full and accurate performance of such respective obligations.

10.3 Force Majeure. If and to the extent that either Party is prevented or delayed by a Force Majeure Event from performing any of its obligations under this Agreement and promptly so notifies in writing the other Party, specifying the matters constituting such Force Majeure Event, together with such evidence in verification thereof as it can reasonably give and specifying the period for which it is estimated that the prevention or delay will continue, then the Party so affected shall be relieved of liability to the other for failure to perform or for delay in performing such obligations (as the case may be), but shall nevertheless use its commercially reasonable efforts to resume full performance thereof.

10.4 Arbitration. All disputes arising out of or in connection with this Agreement shall be settled under the Rules of Arbitration of the International Chamber of Commerce (ICC) in force at the time of submitting the request of arbitration, by one arbitrator appointed in accordance with the said rules. The seat of the arbitration shall be Madrid, Spain. The language of the arbitration shall be English.

10.5 Notices. All notices required or permitted hereunder shall be in writing addressed to the Parties at their respective addresses as set forth below, unless another address shall have been designated:

If to Faes, to:

[**]

Faes Farma, S.A.

Vía de los Poblados 3

28033 Madrid, Spain

With a copy to (which shall not constitute notice):

[**]

Faes Farma, S.A.

Avenida de Autonomía 10

Leioa, Bizkaia, Spain

If to Marathon, to:

Marathon Pharmaceuticals, LLC

1033 Skokie Boulevard

Suite 600

Northbrook, IL 60062

Attn: [**]

will be delivered by hand, by nationally recognized overnight courier, by registered or certified mail, postage prepaid or by facsimile, with confirmation sheet. Any and all notices to be given hereunder shall be deemed delivered on the first business day following delivery by hand, one (1) business day following delivery to a nationally recognized overnight courier for overnight delivery to the recipient and five (5) Business Days following deposit in registered or certified mail as aforesaid.

10.6 Entire Agreement. This Agreement, together with that certain Confidentiality Agreement, dated March 25, 2015 (the "**CDA**"), constitute the entire agreement of the Parties and supersede all prior representations, proposals, discussions, and communications, whether oral or in writing. This Agreement, together with the CDA, may be modified only through a writing signed by the Parties.

10.7 Severability. If any provision of this Agreement shall be held invalid or unenforceable, such provision shall be deemed deleted from this Agreement and replaced by a valid and enforceable provision, which so far as possible, achieves the Parties' intent in agreeing to the original provision. The remaining provisions of this Agreement shall continue in full force and effect.

10.8 Remedies. Each Party agrees that his, her or its obligations hereunder are necessary and reasonable in order to protect the other Party and the other Party's business, and expressly agrees that monetary damages would be inadequate to compensate the other Party for any breach of any covenant or agreement set forth herein. Accordingly, each Party agrees and acknowledges that any such violation or threatened violation of this Agreement (including, but not limited to, Supply Failures as provided for in Section 6.4) will cause irreparable injury to the other Party, and

that, in addition to any other remedies that may be available, in law, in equity, or otherwise, the other Party shall be entitled to seek specific performance for any breach or threatened breach, and to obtain injunctive relief against the threatened breach of this Agreement or continuation of any such breach, without the necessity of proving actual damages. No remedy provided for in this Agreement shall limit (or be construed as limiting) the aggrieved Party's right to any other remedies it may have under this Agreement or in Law, including, without limitation, the recovery of damages for breach of this Agreement, provided however that the limitations on claimable damages under this Agreements agreed in Sections 10.10 and 10.11 shall always apply, except as expressly provided in this Agreement.

10.9 No Waiver. Failure to enforce any provision of this Agreement shall not constitute a waiver of any term or condition hereof.

10.10 Indemnification by Faes. Faes shall indemnify, defend and hold harmless Marathon and its Affiliates and subsidiaries from and against all losses and liabilities and all damages, expenses, costs, and fees, including reasonable attorneys' fees (collectively, "**Losses**"), including, but not limited to, Losses arising from any claim, suit, action or proceeding (each a "**Claim**") brought against Marathon or its Affiliates or subsidiaries by a Third Party, to the extent resulting or arising from any breach by Faes of any representation, warranty, covenant or agreement in this Agreement; provided, however, that Faes shall not be liable under any circumstance to Marathon or its Affiliates or subsidiaries or to any other Third Party for any loss of profit ("*lucro cesante*"), special, consequential, incidental, punitive or indirect Losses arising from or relating to (a) any breach or inaccuracy of Faes' representations or warranties in this Agreement, (b) any breach by Faes of its obligations, undertakings or covenants under this Agreement and (c) any simple negligence in performing its obligations, undertakings or covenants under this Agreement, regardless of any notice of the possibility of such Losses; provided further, however, that the Parties expressly acknowledge and agree that Losses incurred by Marathon involving the payment of monies to a Third Party as a result of Faes' breach of any of its representations, warranties, covenants or agreements in this Agreement (including those described in clauses (a) through (c) above)) shall not constitute (or be deemed to constitute) loss of profit ("*lucro cesante*"), special, consequential, incidental, punitive or indirect Losses for purposes of the exclusion in the preceding proviso.

10.11 Indemnification by Marathon. Marathon agrees to indemnify, defend and hold harmless Faes and its Affiliates and subsidiaries from and against all Losses, including, but not limited to, Losses arising from any Claim brought against Faes or its Affiliates or subsidiaries by a Third Party, to the extent resulting or arising from any breach by Marathon of any representation, warranty, covenant or agreement in this Agreement; provided, however, that Marathon shall not be liable under any circumstance to Faes or its Affiliates or subsidiaries or to any other Third Party for any loss of profit ("*lucro cesante*"), special, consequential, incidental, punitive or indirect Losses arising from or relating to (a) any breach or inaccuracy of Marathon's representations or warranties in this Agreement, (b) any breach by Marathon of its obligations, undertakings or covenants under this Agreement and (c) any simple negligence in performing its obligations, undertakings or covenants under this Agreement, regardless of any notice of the possibility of such Losses; provided

further, however, that the Parties expressly acknowledge and agree that Losses incurred by Faes involving the payment of monies to a Third Party as a result of Marathon's breach of any of its representations, warranties, covenants or agreements in this Agreement (including those described in clauses (a) through (c) above)) shall not constitute (or be deemed to constitute) loss of profit ("*lucro cesante*"), special, consequential, incidental, punitive or indirect Losses for purposes of the exclusion in the preceding proviso.

10.12 Termination for Breach. In addition to the termination rights provided for in Section 6.12(b), each Party shall have the right to terminate this Agreement in its entirety immediately upon written notice to the other Party is the other Party materially breaches its material obligations under this Agreement and, after receiving written notice under this Section 10.12 identifying such material breach in reasonable detail, fails to cure such breach within [**] calendar days after its receipt of such notice (or within [**] calendar days after its receipt of such notice in the event such breach is solely based upon the breaching Party's failure to pay any undisputed amounts due hereunder if such breaching Party fails to cure such breach within such [**] day period); provided, however, that if the Party alleged to in breach disputes such breach in good faith by written notice to the other Party within the applicable cure period (*i.e.,* within the [**]day or, if applicable, [**]day period referred to above), then the Party alleged to be in breach shall not be deemed in breach and the non-breaching Party shall not have the right to terminate this Agreement pursuant to this Section 10.13 unless and until it has been determined in accordance with Section 10.4 that this Agreement was in fact so materially breached and the breaching Party fails to cure such breach within [**] calendar days after such determination. Any abuse or bad faith use by the breaching Party of the provisions of this Section 10.12 in order to avoid the termination of this Agreement hereunder shall be taken into account when determining the amount of damages to be paid by the breaching Party to the non-breaching Party as a result of such termination of this Agreement.

10.13 Expenses. Each Party shall bear its own expenses (including, but not limited to, legal, investment banker, accountant, financial advisor fees and expenses) in connection with this Agreement and the transactions contemplated hereby.

Remainder of page intentionally left blank.

IN WITNESS WHEREOF, the Parties have executed this Agreement individually or through their duly authorized representatives.

FAES FARMA, S.A

By: /s/ Gonzalo Lopez Casanueva
Its: General Manager

MARATHON PHARMACEUTICALS, LLC

By: /s/ Patrick J. Morris
Its: EVP of Legal Affairs and Mergers & Acquisitions and General Counsel

Appendix 1
IND Materials

3.2.P.3.1	Manufacture(s)
3.2.P.3.2	Batch Formula
3.2.P.3.3	Description of Manufacturing Process and Process Controls
3.2.P.3.4	Controls of Critical Steps and Intermediates
3.2.P.4.1	Excipient Specifications
3.2.P.4.2	Analytical Procedures
3.2.P.4.5	Excipients of Human or Animal Origin
3.2.P.4.6	Novel Excipients
3.2.P.5	Control of Drug Product
3.2.P.5.1	Specification(s)
3.2.P.5.2	Analytical Procedures
3.2.P.5.3	Validation of Analytical Procedures
3.2.P.5.4	Batch Analyses
3.2.P.5.5	Characterization of Impurities
3.2.P.6	Reference Standards or Materials
3.2.P.7	Container Closure System
3.2.P.8.1	Stability Summary and Conclusion
3.2.P.8.3	Stability Data

Appendix 2
Faes' Hourly Rates

Faes' Hourly Rates	€[**] Euros) Per Hour

Amendment to Exclusive License and Supply Agreement

THIS AMENDMENT ("Amendment") to the Exclusive License and Supply Agreement dated as of May 12th, 2015 is entered into as of November 1, 2015 (the "Amendment Effective Date") by and between Faes Farma, S.A. ("Faes") and Marathon Pharmaceuticals, LLC ("Marathon").

WHEREAS, Marathon and Faes are parties to that certain Exclusive License and Supply Agreement dated as of May 12, 2015 (the "Agreement"); and

WHEREAS, Marathon and Faes wish to modify the Agreement.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged by each party hereto to the other, both parties mutually agree as follows:

1. Section 6.10 of the Agreement shall be replaced in its entirety by the following:

   6.10 Audits and Facility Access. During the Manufacturing Term, Faes shall allow, during regular business hours and on reasonable prior notice, Marathon's quality assurance, quality control, compliance and other relevant personnel (including Marathon's consultants provided they are under the same confidentiality obligations as Marathon regarding Faes confidential information), to audit the Facilities and related documentation and the Manufacture of Finished Product to be Manufactured and supplied under this Section 6 [**] without cause and additional times per Calendar Year as necessary for cause (each, an "Audit"). The purpose of any such Audit shall solely be to assess compliance with applicable cGMPs and Laws. Furthermore, Faes will allow inspectors from the FDA and other relevant Governmental Entities in the Territory to perform required inspections of such Facilities and related documentation with respect to the Finished Product. Faes shall, without delay, inform Marathon of any such proposed or unannounced FDA or other such Governmental Entity inspections of such Facilities. Faes agrees to permit one or more qualified representative(s) of Marathon to be present on site during any such FDA or other such Governmental Entity inspections pertaining to the Finished Product. Faes shall, without undue delay, provide a summary report of the results of any such FDA or other such Governmental Entity inspection to Marathon. Faes shall, without delay, notify Marathon of any FDA or other such Governmental Entity request for samples of the Finished Product or the API, as applicable.

2. Except as otherwise modified herein, the Agreement will remain in full force and effect.

3. This Amendment shall be governed by and construed by the choice of law from the Agreement.

** ** **

ACKNOWLEDGED, ACCEPTED, AND AGREED TO:

MARATHON PHARMACEUTICALS, LLC

By: /s/ Mr. Patrick J. Morris
Name: Mr. Patrick J. Morris
Title: EVP of Legal Affairs and General Counsel
Date: November 30, 2015

FAES FARMA, S.A.

By: /s/ Mr. Gonzalo Lopez Casanueva
Name: Mr. Gonzalo Lopez Casanueva
Title: General Manager
Date: November 20th 2015

Exhibit 10.2

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

COMMERCIAL MANUFACTURING AGREEMENT

THIS MANUFACTURING AGREEMENT (the "Agreement") is made and entered into this 18th day of September, 2015 (the "Effective Date"), by and between **AAIPharma Services Corp.,** having a place of business at 2320 Scientific Park Drive, Wilmington, NC 28405 ("AAIPharma") and **Marathon Pharmaceuticals, LLC** having a place of business at 1033 Skokie Blvd., Suite 600, Northbrook, IL 60062 USA ("Company"). AAIPharma and Company, as used herein, may be referred to, collectively, as "Parties" and individually as a "Party".

Recitals

WHEREAS, subject to the terms and conditions contained in this Agreement, Company desires to engage the services of AAIPharma to Manufacture the Products (each as defined below) for subsequent commercial distribution by Company.

WHEREAS, AAIPharma is willing to undertake such Manufacture for Company according to the terms and conditions provided for in this Agreement.

NOW, THEREFORE, for and in consideration of the foregoing premises and of the mutual covenants of the Parties hereinafter set forth, the Parties hereto agree as follows:

ARTICLE 1

DEFINITIONS

The following words, terms and phrases, when used herein, shall have the following respective meanings:

1.1 "AAIPharma" shall have the meaning set forth in the preamble.

1.2 "AAIPharma Indemnified Parties" shall have the meaning set forth in Section 8.2.

1.3 "Act" shall mean the United States Food, Drug, and Cosmetic Act (21 U.S.C. 301 et seq.), as amended from time to time, and the regulations promulgated thereunder.

1.4 "API" shall mean the active pharmaceutical ingredient with respect to each Product.

1.5 "Applicable Law(s)" shall have the meaning set forth in Section 3.3.

1.6 "Batch" shall mean a specific quantity of material produced in a contiguous process or series of processes that is expected to be homogeneous within specified limits. The Batch size for each Product is set forth in Exhibit A attached hereto and incorporated herein by reference.

1.7 "cGMP" or **"GMP"** shall mean the recognized pharmaceutical regulations and requirements of regulatory authorities such as those defined by the U.S. FDA's regulations at 21CFR Parts 210 and 211, those defined by Eudralex, "The Rules Governing Medicinal Products in the European Union," and specifically Volume 4, "Guidelines for Good Manufacturing Practices for Medicinal Products for Human and Veterinary Use" and applicable Annexes (Directives 2001/83/EC and amendments including Directives 2003/94/EC dated October 2003 and 2004/27/EC dated March 2004 and/or others that may be appropriate for the particular project) and as may be amended from time to time.

1.8 "Commercialize" or **"Commercialization"** shall mean, with respect to a Product, the marketing, promotion, sale and distribution of such Product.

1.9 "Company" shall have the meaning set forth in the preamble.

1.10 "Company Indemnified Parties" shall have the meaning set forth in Section 8.1**.**

1.11 "Firm Commitment" shall have the meaning set forth in Section 4.1**.**

1.12 "Firm Forecast" shall have the meaning set forth in Section 4.1.

1.13 "Firm Order" shall have the meaning set forth in Section 4.2(b).

1.14 "Indemnification Claim" shall have the meaning set forth in Section 8.3(a).

1.15 "Initial Term" shall have the meaning set forth in Section 9.1**.**

1.16 "Launch" means, with respect to a Product, the first commercial product shipped from AAIPharma's site.

1.17 "Long-Term Forecast" shall have the meaning set forth in Section 4.1**.**

1.18 "Losses" shall have the meaning set forth in Section 8.1.

1.19 "Manufacture"/"Manufacturing" shall mean the manufacture, processing, packaging, labeling (subject to Section 3.7), quality control and testing of the Products performed prior to their delivery by AA1 Pharma in accordance with the terms of this Agreement.

1.20 "Marketing Authorizations" shall mean the United States new drug application or abbreviated new drug application, as applicable, for the Product(s).

1.21 "Master Batch Record" shall mean the batch record as mutually agreed upon by the Parties.

1.22 "Material Change" shall have the meaning set forth in Section 3.3.

1.23 "Minimum Order Requirement" shall have the meaning set forth in Section 4.2(a).

1.24 "Product(s)" shall mean those products described in Exhibit A, as the same may be amended from time to time upon mutual agreement of the Parties; provided, however, that no product

shall become a Product until such time as AAIPharma has successfully completed the registration batches for such product to Company's reasonable satisfaction.

1.25 "Purchase Prices" shall have the meaning set forth in Section 5.1.

1.26 "Quality Agreement" shall have the meaning set forth in Section 6.5.

1.27 "Raw Materials" shall mean any excipient and component materials used to Manufacture the Products, but excluding the API.

1.28 "Raw Material Costs" shall have the meaning set forth in Section 5.2.

1.29 "Recall" shall have the meaning set forth in Section 6.4(b).

1.30 "Release To The Client" shall mean AAIPharma has: i) manufactured and/or packaged and/or labeled the Product according to the Master Batch Record; ii) fulfilled its testing/analytical obligations as further set forth herein; and iii) all manufacturing and testing services performed by AAIPharma have been reviewed and approved by AAIPharma's Quality department.

1.31 "Renewal Period" shall have the meaning set forth in Section 9.1.

1.32 "Services" shall mean certain pharmaceutical development services in addition to manufacturing services including, for example, analytical method development and analysis, stability services, clinical packaging, validation services, quality assurance and regulatory consulting provided by AAIPharma.

1.33 "Specifications" shall mean the specifications for the Products agreed upon by the Parties and included in the Master Batch Record, an example of which is set forth in Exhibit B attached hereto and incorporated herein by reference.

1.34 "Term" shall have the meaning set forth in Section 9.1.

1.35 "Territory" shall mean the United States, its territories and possessions.

ARTICLE 2

LICENSE GRANT TO AAIPHARMA TO MANUFACTURE PRODUCT

2.1 Grant. Company hereby grants to AAIPharma during the Term of this Agreement, on a Product-by-Product basis, a nonexclusive, royalty-free right to Manufacture the Products in the Territory and to use any and all of Company's licenses, trademarks, regulatory data and/or technical information, know how and Confidential Information of Company related to the Products for the purpose of AAIPharma carrying out its obligations hereunder, subject to the conditions of this Agreement.

2.2 Marketing Authorizations. Company shall maintain the Marketing Authorizations in full force and effect at all times. Upon request by Company, AAIPharma shall use commercially reasonable efforts to assist Company in connection therewith; provided that, in exchange, Company will pay AAIPharma its standard fees and expenses therefor.

ARTICLE 3

MANUFACTURING

3.1 Engagement.

(a) During the Term of this Agreement and subject to the terms and conditions set forth herein, Company agrees to exclusively purchase from AAIPharma, and AAIPharma agrees to exclusively manufacture and supply, one hundred percent (100%) of Company's requirements for each Product for Commercialization in the Territory. Notwithstanding the foregoing, Company shall be entitled, at its sole cost and expense, to qualify other manufacturer(s) to manufacture Products solely for the purpose of such manufacturer(s) supplying Company with quantities of Product that AAIPharma is unable to supply (i) in breach of this Agreement, or (ii) during events of force majeure. Following the Initial Term, AAIPharma may notify Company if such exclusivity commitment would prevent AAIPharma from providing services to a third party. The Parties shall negotiate in good faith mutually acceptable revised terms including but not limited to termination of exclusivity or compensation to AAIPharma for the lost opportunity to provide services to such third party. If the Parties fail to agree on terms within [**] days of such notification, either Party shall have the right to terminate this Agreement effective eighteen (18) months following the [**] day negotiation period.

(b) Notwithstanding the foregoing, to the extent Company intends to Commercialize a Product in a jurisdiction outside the Territory, for purposes of such Product only, the term "Territory" shall be expanded to include such jurisdiction provided that AAIPharma agrees in writing and AAIPharma is or becomes compliant with all laws, regulations and other legal and industry requirements applicable to the Manufacture of such Product for subsequent Commercialization of such Product in such jurisdiction. In the event AAIPharma is unwilling or unable to supply Product to a jurisdiction outside the Territory that Company intends to Commercialize, then Company may qualify other manufacturer(s) to manufacture Product solely for that jurisdiction outside the Territory and Company's obligation to purchase Product exclusively from AAIPharma shall be waived for that jurisdiction.

3.2 Manufacture of Commercial Drug Product. Subject to the terms and conditions contained herein, AAIPharma shall Manufacture, hold, handle and prepare for shipment all Product Manufactured pursuant to this Agreement (a) in accordance with this Agreement and the Quality Agreement, and (b) in material compliance with cGMP applicable to the Manufacturing of the Product to be Commercialized in the Territory.

3.3 AAIPharma Changes to Manufacturing Process. Except as required by applicable federal, state, provincial or local law and/or respective regulations as established by the FDA and/or other regulatory authority (collectively, "Applicable Law(s)"), or cGMP, AAIPharma shall not Materially Change the Manufacturing process of a Product or change the facility where a Product is Manufactured that requires a change to a Marketing Authorization without the prior written consent of Company, which consent shall not be unreasonably withheld or delayed. AAIPharma shall notify Company of all material changes, including Material Changes required by

Applicable Law, as soon as practicable after AAIPharma learns of such change. A "Material Change" is one that requires a submission to the FDA, EU, or other applicable regulatory authority.

3.4 Company Requested Changes. Company shall inform AAIPharma in writing of any proposed modifications to the Specifications or the Manufacturing process. Any proposed change shall require AAIPharma's prior written consent, which consent shall not be unreasonably withheld or delayed. AAIPharma shall make changes it agrees to as promptly as practicable; provided, however, that such changes comply with Applicable Law, cGMP and the Marketing Authorizations.

3.5 Costs of Changes. Unless otherwise agreed by the Parties, any and all direct costs associated with changes requested by AAIPharma and changes required by Applicable Law that apply generally to AAIPharma's facility where the applicable Manufacturing occurs shall be borne by AAIPharma; provided however, in the event Applicable Law imposes a registration fee (such as GDUFA) or similar fee on AAIPharma's cGMP facilities, and the fee relates to AAIPharma's services hereunder, the Parties shall determine in good faith an equitable portion of such fee to be paid by Company. Unless otherwise agreed by the Parties, any and all direct costs associated with all other changes, including, without limitation, changes requested by Company, changes required by Applicable Laws that apply specifically to a Product, and changes required by a change to a Marketing Authorization, shall be borne by Company (collectively, the "Other Changes"). If the change is an Other Change, (i) the Purchase Prices shall be adjusted by the change in AAIPharma's cost of Manufacture of the Product caused by such Other Change, plus an amount necessary to maintain AAIPharma's profit margin on such, and (ii) Company shall reimburse AAIPharma for costs, expenses or losses associated with write-offs, obsolescence and/or destruction of any work in process or finished inventory resulting from any such Other Change.

3.6 Notification and Approval of Changes. Company shall have sole responsibility for obtaining any and all necessary regulatory approvals from the relevant regulatory agencies in the Territory for changes to the Specifications and the Marketing Authorizations and for reporting any changes to such Specifications and the Marketing Authorizations to the relevant regulatory agencies in the Territory as appropriate. Upon request by Company, AAIPharma shall use commercially reasonable efforts to assist Company in obtaining any such approvals; provided that Company will pay AAIPharma its standard fees and expenses therefor.

3.7 Labeling. Company shall be responsible for the labeling to be used on each Product and the packaging thereof, including any changes to such labels; provided that Company shall ensure that all such labeling complies with Applicable Laws. AAIPharma shall use the specified labeling (and only such labeling) on the Products, and shall not use such labeling on any other product. Any Company-directed change to a Product label shall be implemented by AAIPharma as soon as reasonably practicable following AAIPharma's receipt of written notification of such label changes. Company shall reimburse AAIPharma for costs incurred in connection with any such label changes, including without limitation, the costs of obsolescence of goods-in-process, packaging materials

and supplies and finished goods not suitable for Commercializing in the Territory due to such label changes.

3.8 Finished Product Release. AAIPharma will provide Company with manufacturing documents as are necessary for Company to release each lot of Product for human use. Company shall be responsible for the final release of Product for human use.

3.9 Raw Materials and API. AAIPharma shall purchase at its own expense and for its own account all Raw Materials, packaging components and other items of any nature whatsoever that AAIPharma may use to Manufacture the Products. Except as otherwise agreed to between the Parties, all right, title and interest in and to these items, and in and to all work-in-process incorporating these items, shall remain the sole property of AAIPharma until Products incorporating such items are delivered for shipment to Company. However, the total cost of changing the source and/or type of Raw Materials shall be at the sole cost of Company. Company shall supply to AAIPharma at its own expense and for its own account all API to be used in the Manufacture of Products hereunder, and such API shall remain the sole property of Company. AAIPharma shall have no liability for lost or damaged API unless caused by its negligence or intentional misconduct. If such losses, in an annual reconciliation, lead to actual yields below [**] percent ([**]%) of theoretical, AAIPharma shall issue a credit to Company for the lesser of (a) an amount equal to Company's then current replacement cost of the API, or (b) the amount AAIPharma would have charged Company for the amount of Product the lost quantity of API would have yielded. As such, if Company desires to insure its API, Company should do so under its own appropriate insurance policy. Company shall provide AAIPharma with documentation of its API cost at least annually on or before the anniversary of the Effective Date.

ARTICLE 4

FORECASTS, ORDERS, DELIVERY AND ACCEPTANCE

4.1 Forecasting. Company shall provide to AAIPharma a written good faith forecast estimating Company's quarterly requirements (broken out on a month-to-month basis) of each Product for each of the first [**] calendar quarters during the Term at least [**] months prior to Launch of each Product. In addition, within [**] weeks after the start of each quarter (i.e. [**]) during the Term, Company shall provide AAI Pharma with an updated rolling [**] month forecast estimating Company's requirements (broken out on a month-to-month basis) of the Product that shall cover the succeeding [**] calendar month period (or the period until the expiration of the Term, if shorter) (each such forecast, a "Long-Term Forecast"). Except as set forth in Sections 4.2(a) and 4.2(b), the Long-Term Forecast shall not be binding on either Party, but for the first [**] calendar months of a Long-Term Forecast, which shall be a "Firm Commitment" with respect to the Product, and but for the [**] calendar months of a Long-Term Forecast, which shall be a "Firm Forecast" with respect to the Product.

4.2 Firm Commitments.

(a) Each Firm Commitment shall be a binding commitment for the quantities of each Product forecast for the first [**] calendar months of the Long-Term Forecast. The quantity of each Product specified in any Firm Commitment for delivery to, and purchase by, Company in any calendar quarter shall not be less than [**] percent ([**]%) of the quantities forecasted for such quantities when it was the applicable Firm Forecast (the "Minimum Order Requirement").

(b) With respect to each Firm Commitment, Company shall submit to AAIPharma binding written purchase orders (a "Firm Order") no later than [**] days prior to the requested delivery dates confirming the quantity of each Product ordered (which shall be in full Batch quantities), the requested delivery dates, and such other information as AAIPharma may find reasonably necessary to Manufacture the ordered Products. AAIPharma will confirm the requested delivery dates within [**] business days of receipt of a Firm Order.

(c) If Company fails to order and purchase the Minimum Order Requirement, then within [**] days following the end of the quarter in which the Minimum Order Requirement was not met, Company shall pay to AAIPharma the difference between: (i) the Purchase Price for the applicable Minimum Order Requirement, and (ii) the Purchase Price that was paid by Company for the quantity ordered.

Furthermore, Company agrees that purchases may be made by AAIPharma of the Raw Materials, packaging components and other items to satisfy the production requirements for the Long-Term Forecast. In such circumstances, if such Raw Materials, packaging components and other items are not included in finished Products purchased by Company within [**] months after such purchases have been made (or such longer period as the Parties may have agreed to), Company will pay to AAIPharma its costs thereof and, in the event such Materials are incorporated into Products subsequently purchased by Company, Company will receive credit for any of such costs previously paid to AAIPharma by Company.

(d) AAIPharma shall Manufacture and prepare for shipment the quantity of a Product specified in the Firm Commitment and related purchase orders. Notwithstanding the foregoing, with respect to a Product, in no event shall AAIPharma be required in any calendar quarter to deliver more than [**] percent ([**]%) of the quantities in the applicable Firm Forecast, but AAIPharma shall use its commercially reasonable and good faith efforts to deliver quantities in excess of [**]% of the applicable Firm Forecast. The Firm Commitments shall be made available for shipment in accordance with Section 4.4.

4.3 Changes in Orders. AAIPharma shall exercise its commercially reasonable efforts to comply with any proposed amendments to accepted Firm Orders that Company may request, but AAIPharma shall not be liable in any way for its inability to do so. Firm Orders may be amended only by mutual agreement of the Parties and such amendments shall not affect the Minimum Order Requirement.

4.4 Delivery. AAIPharma shall use commercially reasonable efforts to make Product available for shipment within [**] business days of the delivery date requested in the applicable Firm Order. Company shall pay all crating, skidding, rigging, customs, freight, shipping, insurance and common carrier charges on all shipments in connection with Company's chosen method of shipment of the Product. All Product(s) shall be shipped EX WORKS (Incoterms 2010) AAIPharma's manufacturing facility. Title and risk of loss of Product shall pass to Company at the time the Products are placed on AAIPharma's loading dock at Company's disposal, not cleared for export and not loaded on any collecting vehicle. Company shall be responsible for arranging the shipment of the Product(s) from AAIPharma's manufacturing facility to its final destination (and storage charges shall be imposed [**] days after notice to Company that Product is available for shipment); provided, however, that Company must provide AAIPharma with reasonable evidence (e.g. a copy of the current DEA registration for the destination, when applicable) that such destination is authorized to handle the Product. Notwithstanding anything to the contrary in this Agreement, Company acknowledges and agrees that AAIPharma shall have no obligation to release Product for shipment to any destination for which Company has not provided adequate evidence of authorization as required in this Section 4.4. AAIPharma shall not be liable to Company for Product which is damaged or lost while in possession of a common carrier, and it shall be Company's responsibility to recover any and all damage directly from such common carrier.

4.5 Inspection, Acceptance and Rejection of Delivered Products.

(a) Company will have [**] days from receipt by Company to inspect and test Products for noncompliance with the applicable Specifications (the "Inspection Period").

(b) Except as provided in Section 4.5(c), Company shall give written notice if it intends to reject a Batch(es) of Product(s) - for not complying with the Specifications - within [**] days after the Inspection Period expires; otherwise such Batch(es) shall be deemed accepted.

(c) If, after the Inspection Period, Company first discovers that a Batch(es) of Product(s) do not comply with the applicable Specifications, then Company shall so notify AAIPharma if it intends to reject such Batch(es) within [**] days after such discovery; otherwise such Batch(es) shall be deemed accepted. AAIPharma will only be responsible for Batch(es) of Product(s) rejected after the Inspection Period solely to the extent that AAIPharma is responsible for said non-conformity.

(d) Notwithstanding anything to the contrary herein, AAIPharma shall not be responsible for damages to Product during shipment, and in no event shall AAIPharma be responsible for noncompliance with Specifications for Product that met Specifications at time of Release To The Client or from non-conformities that result from a deficiency or change in the API utilized in such Batch(es) of Product(s) or a defect in the Specifications for the Products.

(e) In the event that Company rejects Product(s) as provided in this Agreement, AAIPharma shall use commercially reasonable efforts (but within [**] days after AAIPharma's receipt of Company's notice of noncompliance) to replace the defective Product(s) or give notice

that it disagrees with the rejection. If Company and AAIPharma do not agree whether the Product(s) failed to meet applicable Specifications at the time of Release To The Client, such Products shall be submitted for testing to an independent laboratory or other authority of national reputation acceptable to both Parties for the purpose of determining the results. Any determination by such authority shall be final and binding upon the Parties hereto. If Company's rejection is substantiated by the authority, AAIPharma shall pay the expenses associated with such analyses; otherwise Company shall pay such expenses and purchase the Product.

4.6 Non-Conforming Product(s). Notwithstanding any other provisions of this Agreement, Company agrees, if so requested by AAIPharma, to return to AAIPharma any Product(s) that fail to meet Specifications or otherwise to dispose of such Product(s) as AAIPharma may direct, each at AAIPharma's expense.

ARTICLE 5

PRICE, TERMS OF PAYMENT

5.1 Purchase of Product(s). The initial prices to be paid for the Products by Company to AAIPharma shall be set forth in Exhibit A attached hereto and incorporated herein by reference (the "Purchase Prices"). The Purchase Prices are in United States dollars, and are exclusive of applicable taxes. Company shall be responsible for the payment of any and all taxes applicable to the Products and Services described herein.

5.2 Price Change; Notice. AAIPharma may increase the Purchase Prices during the Term by [**]. Upon request by Company, AAIPharma shall provide reasonable documentation that reflects the increase in cost of Raw Material Costs. AAIPharma shall provide written notification of any annual increase in the Purchase Prices prior to the January 1st effective date of the increase in Purchase Prices, or as increases in the cost of Raw Materials occur, as applicable.

5.3 Invoices. AAIPharma shall provide invoices to Company for the Product(s) upon each Release To The Client (e.g. finished bulk, finished packaged, or finished packaged and labeled), and Company shall pay each such invoice, in United States dollars, within [**] days after the date of each invoice regardless of when or whether Company has arranged for shipment of the Product(s) to its final destination. Company shall make no setoff or deduction of any kind from any payments due to AAIPharma unless Company receives written authorization from AAIPharma authorizing such setoff or deduction. Undisputed invoice balances not remitted within [**] days of the date of each invoice shall be subject to a [**] percent ([**]%) per month interest charge. Should any part of the invoice be in dispute, Company shall pay the balance of the undisputed amount according to the terms and conditions described herein while said dispute is being resolved. Should payment of undisputed amounts not be received within [**] days of invoice date, and after [**] days notice to Company, the payment shall be deemed in default and AAIPharma reserves the right to cease all work and pursue collection activities. In the event of default in payment, Company shall be responsible for all collection fees and expenses incurred by AAIPharma, including reasonable attorney's fees.

ARTICLE 6

REGULATORY MATTERS; RECORDS

6.1 Annual Review and Stability Testing. If listed in Exhibit A, AAIPharma will conduct an annual product review for the Products and upon completion of such review will forward a copy to Company. The Parties agree that AAIPharma's Manufacturing process and the Purchase Prices do not include stability testing or any other work not specifically set forth herein or in an Exhibit hereto. Stability testing services and other services shall be provided at the then current AAIPharma rates for such services.

6.2 Access to AAIPharma's Facilities by Company Representatives for Quality Audit. During regular business hours and mutually agreed upon times, Company may review the records of AAIPharma and observe the manufacturing processes relating to the Services performed and expenses incurred to assure compliance with all provisions of this Agreement. Such review must be completed in not more than [**] business days and shall be offered to Company by AAIPharma [**] and may be more pursuant to cause. Subsequent reviews during the same calendar year or such reviews that cannot be completed in [**] business days will be at Company's sole cost and expense, at AAIPharma's then current rates. Company shall also be provided an invoice for any incidental expenses AAIPharma incurs resulting from such review. Company's rights in this Section 6.2 shall be subject to compliance with AAIPharma's reasonable measures for purposes of confidentiality, safety, and security, and will be further subject to Company's compliance with AAIPharma's premises rules that are generally applicable to all persons at AAIPharma's facilities. Should Company utilize one or more third party(ies) in exercising its rights in this paragraph, Company certifies that such party(ies) shall be subject to an obligation of confidentiality consistent with the obligations of confidentiality required of Company hereunder and such third party(ies) shall be subject to any and all conditions upon Company's rights that are set forth in this Section.

6.3 Inspections by Governmental or Regulatory Authority. AAIPharma shall be responsible for handling and responding to any FDA or other governmental body inspections or inquiries received by Company or AAIPharma regarding the Manufacturing of any Product during the Term. In cases where AAIPharma is required to provide significant Company or Product specific support to such inspections or inquiries, Company agrees to pay AAIPharma for the time required at the then current AAIPharma regulatory support rate. Each Party shall promptly notify the other regarding any such inquiries and provide the other Party copies of any pertinent correspondence from such authorities related to the Product or Services covered in this Agreement. AAIPharma shall provide to Company and any governmental body any information reasonably requested by Company and/or such governmental body concerning any governmental inspection related to any Product (with all information provided to Company being subject to the confidentiality provisions in Section 10.1 herein and with AAIPharma being able to redact any information provided to Company to remove third party confidential information that does not relate to the Products). AAIPharma agrees to notify Company of any regulatory inspection specific to one or more of the Products and shall allow Company to send a representative to the site being audited, however participation in the audit will be at the sole discretion of AAIPharma. Company agrees to fully

cooperate with and assist as requested by AAIPharma in fulfilling the obligations pursuant to this Section 6.3.

6.4 Complaints, Recalls, and Insurance

(a) Complaints. Product complaints received by Company with respect to Product Manufactured by AAIPharma hereunder shall be faxed to AAIPharma within [**] business days after receipt to:

AAIPharma Services Corp.
Attention: Corporate Quality
2320 Scientific Park Drive
Wilmington, NC 28405
Facsimile No.: [**]

As more fully described in the Quality Agreement, AAIPharma shall investigate all complaints directly associated with the Manufacture of Product(s) and shall provide an update every [**] days and a report to Company regarding its investigation and any conclusions. Company shall investigate all other complaints associated with the Product(s).

(b) Recall Procedures. In the event that a recall, withdrawal or field correction of any Product (a "Recall") is initiated, whether by a statutory or regulatory authority in any jurisdiction or by Company, AAIPharma shall reimburse Company for all costs and expenses incurred in procuring or complying with the requirements of such Recall to the extent that such Recall is initiated as a result of AAIPharma's breach of this Agreement (which shall include but not be limited to AAIPharma's noncompliance or nonconformity with the Specifications, GMP, or any Applicable Laws), intentional misconduct, negligence, or defective manufacturing, processing, testing, packing, or storage of Product prior to delivery to Company, and, in addition, AAIPharma shall refund to Company an amount equal to the cost of all API supplied to AAIPharma and incorporated into the recalled Product; but not more than the cost of the Batch(es) or portion of a Batch, prorated. Company shall be responsible for all other costs and expenses associated with a Recall. AAIPharma shall reasonably cooperate with Company in connection with any Recall.

(c) Insurance. At all times while this Agreement is in effect and for [**] years thereafter, AAIPharma and Company shall each:

i maintain general liability insurance (including, without limitation, product liability insurance, liability for property damage, personal injury and contractual liability) with Products/Professional at limits not less than $[**] per occurrence/$[**] aggregate;

ii maintain Workers' Compensation as required by all applicable laws and Employer's Liability coverage with a limit of not less than $[**]; and

iii provide, within [**] days of the other Party's request, Certificates of Insurance verifying insurance limits agreed upon as well as a [**] day Notice of Cancellation or Non-Renewal.

AAIPharma and Company shall each obtain all the insurance policies described in clauses 6.4(c)(i) and (ii) from insurers having A.M. Best ratings of A-VII or higher.

Company shall, at its own cost and expense, obtain and maintain in full force and effect during the Term of this Agreement All Risk Property Insurance, including transit coverage, in an amount equal to full replacement value covering Company's property while it is at AAIPharma's facilities or in transit to or from AAIPharma's facilities. Company shall obtain a waiver from any insurance carrier with whom Company carries All Risk Property Insurance releasing its subrogation rights against AAIPharma. Company shall not seek reimbursement for any property claim, or portion thereof, that is not fully recovered from Company's All Risk Property Insurance policy.

6.5 Quality Agreement. The Parties intend to enter into a quality agreement acceptable to both Parties (the "Quality Agreement") as soon as practicable after the Effective Date. The Quality Agreement will detail the quality and regulatory obligations and responsibilities of the Parties with respect to the Products to the extent these obligations and responsibilities are not fully covered in this Agreement; provided, however, that in the event of conflict between the terms of this Agreement and the Quality Agreement, (i) the provisions of the Quality Agreement will prevail with respect to all matters pertaining to, or governed by, GMP and (ii) in all other respects, the provisions of this Agreement will prevail.

ARTICLE 7

REPRESENTATIONS AND WARRANTIES

7.1 Representations and Warranties of AAIPharma. AAIPharma hereby represents and warrants as follows:

(a) As of Release To The Client, all Product(s) delivered to Company during the Term of this Agreement: (i) shall have been Manufactured by AAIPharma in material compliance with this Agreement, the Quality Agreement, the Marketing Authorizations and cGMP, in each case, as in effect at the time of Manufacture, (ii) assuming compliance by Company with Section 3.7, shall not be adulterated or misbranded within the meaning of the Act, and (iii) shall not have been Manufactured by AAIPharma in violation of any Applicable Law in any material respect.

(b) Upon delivery, AAIPharma shall convey good title to all Product(s) so delivered to Company.

(c) The execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby are within AAIPharma's powers and have been duly authorized by all necessary action on the part of AAIPharma. This Agreement has been duly executed and delivered by AAIPharma and constitutes legal, valid and binding obligations of AAIPharma, enforceable against AAIPharma in accordance with its terms.

(d) The execution, delivery and performance by AAIPharma of this Agreement does not and will not (i) contravene or conflict with the organizational documents of AAIPharma Services Corp., (ii) contravene or conflict with or constitute a violation of any Applicable Laws, or (iii) breach or constitute a default under the provisions of any material contract, agreement or instrument to which it is a party or by which it is bound.

(e) AAIPharma is not debarred and has not and shall not knowingly and intentionally use in any capacity the services of any third person debarred under subsections 306(a) or (b) of the Generic Drug Enforcement Act of 1992.

EXCEPT AS SET FORTH IN THIS SECTION 7.1, AAIPHARMA MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, AND SPECIFICALLY DISCLAIMS ALL SUCH REPRESENTATIONS AND WARRANTIES, INCLUDING, WITHOUT LIMITATION, ANY IMPLIED WARRANTIES OF MERCHANTABILITY, INFRINGEMENT, TITLE OR FITNESS FOR A PARTICULAR PURPOSE OR USE.

7.2 Representations and Warranties of Company. Company hereby represents and warrants as follows:

(a) The execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby are within Company's powers and have been duly authorized by all necessary action on the part of Company. This Agreement has been duly executed and delivered by Company and constitutes legal, valid and binding obligations of Company, enforceable against Company in accordance with its terms.

(b) The execution, delivery and performance by Company of this Agreement does not and will not (i) contravene or conflict with the organizational documents of Company, (ii) contravene or conflict with or constitute a violation of any Applicable Laws, or (iii) breach or constitute a default under the provisions of any material contract, agreement or instrument to which it is a party or by which it is bound.

(c) Company shall comply in all material respects with all Applicable Laws relating to its Commercialization of the Product(s).

(d) To the extent that Company supplies any Raw Materials, or API, or other information to AAIPharma (including packaging and labeling requirements) or engages in manufacturing with respect to any of the Products (either directly or indirectly through a third party), all such Raw Materials, API or other information and formulas will comply with the Specifications and applicable laws, including GMP.

(e) Company represents that to the best of its knowledge, the manufacture or the sale of the Products does not and will not infringe any third party intellectual property rights or other rights and that it is not aware of any patents existing in the Territory in which Company markets or distributes such Products relating in any manner to the Products or any use, method, activity or

application relating thereto which could adversely impact upon or prevent AAIPharma from Manufacturing the Products as contemplated by the terms hereof.

ARTICLE 8

INDEMNIFICATION

8.1 By AAIPharma. AAIPharma hereby indemnifies Company and its directors, officers, employees, Affiliates, stockholders, agents, attorneys, representatives, successors and Permitted Assigns (collectively, the "Company Indemnified Parties") against and agrees to hold each of them harmless from any and all product liability claims associated with the Products, losses, liabilities, obligations, damages, costs and expenses ("Losses") incurred by any Company Indemnified Party as a result of third party claims, actions or proceedings (collectively, "Third Party Claims") to the extent based upon, attributable to or resulting from: (a) any material misrepresentation or material breach of warranty made by AAIPharma in this Agreement, (b) any material breach of any covenant or agreement made or to be performed by AAIPharma pursuant to this Agreement, and (c) the negligence or willful misconduct by an AAIPharma Indemnified Party in connection with this Agreement; except in each case, to the extent such Losses are attributable to Company's material breach of this Agreement or arising from the negligence or willful misconduct of Company.

8.2 By Company. Company hereby indemnifies AAIPharma and its directors, officers, employees, Affiliates, stockholders, agents, attorneys, representatives, successors and assigns (collectively, the "AAIPharma Indemnified Parties") against and agrees to hold each of them harmless from any and all Third Party Claims, including Losses incurred by any AAIPharma Indemnified Party to the extent based upon, attributable to or resulting from the performance of this Agreement and Services hereunder by AAIPharma (including, without limitation, any products liability claims related to Company products) other than for Losses for which AAIPharma is obligated to indemnify the Company Indemnified Parties under Section 8.1 above.

8.3 Indemnification Procedures.

(a) The indemnified Party shall give the indemnifying Party prompt notice of any such claim or lawsuit ("Indemnification Claim") (including a copy thereof) served upon it and shall fully cooperate with the indemnifying Party and its legal representatives in the investigation of any matter the subject of indemnification. The indemnifying Party may enter into a settlement agreement with a claimant but shall not admit liability to a claimant without the prior written permission of the party or parties seeking indemnification, which permission shall not be unreasonably withheld.

(b) The failure of the indemnified Party to give reasonably prompt notice of any Indemnification Claim shall not release, waive or otherwise affect the indemnifying Party's obligations with respect thereto except to the extent that the indemnifying Party can demonstrate actual loss and prejudice as a result of such failure.

8.4 Limitation on Liability. Except as set forth in Section 8.6 (Exceptions), neither Party shall be liable, whether in contract, tort (including negligence) or otherwise, for any punitive,

special, indirect, incidental, consequential or exemplary damages (including lost profit or business interruption even if notified in advance of such possibility) arising out of or pertaining to the subject matter of this Agreement.

8.5 Aggregate Cap. Except as set forth in Section 8.6 (Exceptions), the total aggregate liability of either Party to the other Party arising out of this Agreement shall be limited to the total amounts paid and payable by Company to AAIPharma under this Agreement during the twelve (12) months preceding the event in question. Such liability cap amount does not alter each Party's insurance obligations under Section 6.4(c) (Insurance).

8.6 Exceptions. Sections 8.4 (Limitation on Liability) and 8.5 (Aggregate Cap) shall not apply to the following: (a) a Party's obligations to indemnify the other for Claims under Sections 8.1 and 8.2 (Indemnification); or (b) damages due to a Party's breach of its confidentiality obligations or claims for infringement of proprietary rights.

ARTICLE 9

TERM AND TERMINATION

9.1 Term of the Agreement. Unless earlier terminated in accordance with this Article 9, this Agreement shall take effect and commence on the Effective Date and continue in effect, on a Product-by-Product basis, for five (5) years following Launch of a particular Product (the "Initial Term"). In addition, after the expiration of the Initial Term with respect to a particular Product, this Agreement will automatically renew with respect to such Product for consecutive two (2) year terms (each, a "Renewal Period") unless either of the Parties terminates this Agreement with respect to such Product at the end of the applicable Initial Term or any applicable Renewal Period by providing the other Party with written notice, in the case of Company, at least twelve (12) months, and in the case of AAIPharma, at least eighteen (18) months, prior to the end of the applicable Initial Term or applicable Renewal Period. The Initial Term and all Renewal Periods for each Product shall be collectively referred to herein as the "Term" for such Product.

9.2 Termination. Notwithstanding Section 9.1 herein, this Agreement may be terminated as follows:

(a) immediately upon the delivery of written notice by one Party, if the other Party materially breaches any of the provisions of this Agreement and such breach is not cured within [**] calendar days after receipt of written notice identifying such breach (or if cure has been commenced during such period, if it is not diligently prosecuted to completion); or

(b) immediately upon the delivery of written notice by one Party, if the other Party has been unable to perform its obligations hereunder for one hundred twenty (120) calendar days by reason of force majeure (as defined in Section 12.11).

(c) either Party at its sole option may immediately terminate this Agreement upon written notice, but without prior advance notice, to the other Party in the event that (i) the other Party is declared insolvent or bankrupt by a court of competent jurisdiction; (ii) a voluntary petition of

bankruptcy is filed in any court of competent jurisdiction by such other Party; (iii) ceases or threatens to cease to carry on business, or (iv) this Agreement is assigned by such other Party for the benefit of creditors.

(d) Company may terminate this Agreement as to any Product upon forty-five (45) days' written notice in the event that any governmental agency takes any action, or raises any objection, that prevents Company from importing, exporting, purchasing or selling such Product.

(e) Company may at any time unilaterally terminate this Agreement only with respect to an individual Product if: (i) such individual Product is withdrawn from the market; (ii) Company divests, out-licenses or otherwise disposes of such individual Product to a party other than an Affiliate of Company; provided, however, for greater certainty, that this Subsection 9.2(e) shall not entitle Company to terminate this Agreement in whole or in part in connection with a sale or other disposition of all or substantially of its interest in the Products as a whole or any significant portion thereof; or (iii) such individual Product is found to infringe a third party's Intellectual Property.

Company shall provide to AAIPharma not less than twelve (12) months' advance written notice of such partial termination of this Agreement except where it results from either a market withdrawal at the mandate of a competent authority having jurisdiction or an infringement as in Subsection 9.2(e)(iii) above, in which cases the termination can be effective immediately; provided, however, in respect of Subsection 9.2(e)(ii), Company may provide less than the twelve months advance notice if the acquiring party agrees in writing to purchase the particular individual Product from AAIPharma for the balance of the notice period on the same terms and conditions as contained herein.

Any termination pursuant to this Section 9.2 may be effected with respect to this entire Agreement or with respect to any individual Product or Products, at the discretion of the terminating Party, and shall be effected by delivering written notice of such termination to the other Party and shall be effective upon the date of such written notice unless a later date is specified in such written notice.

9.3 Effect of Termination. Upon termination or expiration of this Agreement, in its entirety or with respect to any particular Product(s):

(a) Cessation of Activities. Except as provided in Section 9.3(c), AAIPharma shall stop the Manufacturing of Products; each Party shall return to the other any Confidential Information of such other Party concerning the Product(s) subject to such termination or expiration.

(b) Payment of Minimum Order Requirement; Company to Take Product. In the event of termination by AAIPharma pursuant to Section 9.2(a), (b), or (c) above, Company shall pay AAIPharma any balance remaining of the Minimum Order Requirement in the same manner as set forth in Section 4.2(c) in the case of a failure to order and purchase the Minimum Order Requirement in any calendar quarter. Company shall, at its option and with respect to any Products that are subject to termination, be permitted to take delivery for any Raw Materials, work-in-process (at AAIPharma's material costs) or finished Product (at prices then in effect under this Agreement).

(c) Firm Orders. If this Agreement is terminated by Company pursuant to Section 9.2(a), at Company's option, Firm Orders with respect to the Product(s) not yet started shall be cancelled, or, if requested by Company in writing, AAIPharma will, with respect to the Product(s) subject to such termination, complete or cause the completion of the Manufacturing of any work-in-process that is subject to a valid and effective Firm Order on the date on which the termination is effective. Once such work-in-process is completed, the resulting Product(s) shall be shipped in accordance with Company's Firm Orders and paid for by Company in accordance with Section 5.3.

9.4 Survival. The Parties agree that the following provisions shall survive the termination of this Agreement; the definitions of Article 1 to the extent such Definitions pertain to terms in surviving provisions, Sections 4.5, 4.6, 6.4, and Articles 5, 7, 8, 9, 10, 11 and 12.

ARTICLE 10

CONFIDENTIALITY AND PUBLIC DISCLOSURE

10.1 AAIPharma will hold in strict confidence, and shall not disclose to any third party without Company's prior written consent, all proprietary or confidential information concerning Product, API and all materials and information provided by Company (collectively, "Company Information"). AAIPharma further agrees that it shall not use Company Information for any purpose other than the Manufacturing of Products for Company under this Agreement.

10.2 Company will hold in strict confidence, and shall not disclose to any third party without AAIPharma's prior written consent, all proprietary or confidential information and materials belonging to AAIPharma ("AAIPharma Information").

10.3 "Confidential Information" shall mean Company Information and AAIPharma Information. Each Party may disclose Confidential Information only to its directors, officers and employees who have need to know Confidential Information for the purposes of this Agreement, and each Party will be responsible for ensuring that all its directors, officers, and employees to whom Confidential Information is disclosed will also observe such obligations of confidentiality and non-use as provided herein.

10.4 The above confidentiality obligation shall not apply or shall cease to apply to any information which the receiving party can demonstrate by documentary proof:

(a) is already in the possession of the receiving party at the time it is disclosed by the disclosing party;

(b) is in the public domain at the time it is disclosed by the disclosing party;

(c) enters the public domain through sources independent of the receiving party and through no fault of the receiving party;

(d) is lawfully obtained by the receiving party without any confidentiality restrictions from a third party who has a right to disclose such information to the receiving party;

(e) has been at any time developed by the receiving party independently of disclosure from the disclosing party.

10.5 Neither Party (nor any of their respective Affiliates) shall issue any press release or make any public announcement with respect to this Agreement and the transactions contemplated hereby without obtaining the prior written consent of the other Party (such consent not to be unreasonably withheld or delayed), except as may be required by Applicable Law upon the advice of counsel and only if the disclosing Party provides the non-disclosing Party with a reasonable opportunity to first review the release or other public announcement, to the extent practicable.

10.6 These confidentiality obligations shall survive termination or expiration of this Agreement for a period of [**] years.

ARTICLE 11

INTELLECTUAL PROPERTY

11.1 AAIPharma further agrees that all Company Information, know-how, data, discoveries and inventions relating to Product and API which result from the Manufacture of Products shall constitute the sole and exclusive property of Company. AAIPharma hereby assigns to Company all right, title and interest throughout the world in and to all inventions (whether or not patentable), processes, techniques, improvements, discoveries and developments discovered and reduced to practice by AAIPharma (collectively, "Project IP") in the course of providing Services which are directly and solely related to the Manufacture of Product hereunder. AAIPharma will, at the expense and the written request of Company, do all reasonable acts and measures and execute all documents as Company may reasonably request to transfer to and vest in Company the ownership and registration of all intellectual property rights that may exist in such Project IP.

11.2 Company acknowledges that AAIPharma possesses certain inventions, processes, techniques, improvements, know-how, trade secrets, discoveries and other intellectual property and other proprietary assets, including drug delivery technologies (hereinafter, "AAIPharma Proprietary Technology") which have been independently developed by AAIPharma. In the event Company chooses to further develop and/or commercialize a technology comprising, in whole or in part, AAIPharma Proprietary Technology, Company will obtain a license from AAIPharma to use such AAIPharma Proprietary Technology. Such license agreement shall be memorialized in a separate agreement to be negotiated in good faith by the Parties.

11.3 Company acknowledges that AAIPharma is in the business of providing services for a variety of organizations other than Company. Accordingly, nothing in this Agreement, with the exception of the exclusivity obligations set forth in Article 3.1 herein, shall preclude or limit AAIPharma from providing services or developing materials for itself or other clients, or from utilizing the general knowledge gained during the course of its performance hereunder to perform similar services for other clients, provided that such provision of services or development of materials do not constitute a breach of confidentiality under Article 10 or the exclusivity obligations set forth in Article 3.1 herein.

ARTICLE 12

MISCELLANEOUS

12.1 Successors and Assigns. Neither Party may assign its rights or obligations under this Agreement without the prior written consent of the other Party; provided, however, that either Party may assign this Agreement, in whole or in part, without such consent, to an Affiliate of such Party or to a Third Party that acquires substantially all of the assets of a Party to which this Agreement relates, upon written notice to the other Party of any such assignment and such Party hereby guarantees the performance of any such Affiliate, and, in the case of a Third Party assignment, such Third Party shall assume the obligations of the assigning Party under this Agreement. No assignment shall relieve any Party of responsibility for the performance of any obligation, which such Party may have or incur hereunder. This Agreement shall be binding upon and inure to the benefit of each of the Parties and each such Party's successors and permitted assigns.

12.2 Notices. Any notice required or permitted under this Agreement shall be in writing, shall specifically refer to this Agreement, and shall be sent by hand, recognized overnight courier, confirmed facsimile transmission, or registered or certified mail service, postage prepaid, return receipt requested, to the following addresses or facsimile numbers of the Parties:

Company:

Marathon Pharmaceuticals, LLC
1033 Skokie Blvd, Suite 600
Skokie, IL 60062 USA
Attn: General Counsel
Fax: [**]

AAIPharma:

AAIPharma Services Corp.
2320 Scientific Park Drive
Wilmington, NC 28405
Attn: Legal Department
Fax: [**]

All notices under this Agreement shall be deemed received (i) upon receipt when sent by hand, (ii) two (2) business days after deposit with a recognized overnight courier, (iii) upon confirmation of delivery when sent by facsimile, and (iv) five (5) business days after deposit in registered or certified mail service. A Party may change its contact information immediately upon written notice to the other Party in the manner provided in this Section.

12.3 Waiver. No delay on the part of AAIPharma or Company in exercising any right, power or privilege hereunder shall operate as a waiver thereof, nor shall any waiver on the part of either Party of any right, power or privilege hereunder operate as a waiver of any other right, power or privilege hereunder, nor shall any single or partial exercise of any right, power or privilege

hereunder preclude any other or further exercise thereof or the exercise of any other right, power or privilege hereunder. Any provision of this Agreement may be waived if, and only if, such waiver is in writing and signed by the Party against whom the waiver is to be effective.

12.4 Entire Agreement. This Agreement and the Quality Agreement constitute the entire agreement between the Parties with respect to the subject matter hereof and supersede all prior agreements, understanding and negotiations, both written and oral, between the Parties with respect to the subject matter of this Agreement.

12.5 Amendment. This Agreement may be modified or amended only by written agreement of the Parties hereto.

12.6 Counterparts. This Agreement may be executed by facsimile and in any number of counterparts, each of which shall be deemed an original but all of which together shall constitute a single instrument. This Agreement may be executed on signature pages exchanged by facsimile, in which event each Party shall promptly deliver to the others such number of original executed copies as the others may reasonably request.

12.7 Governing Law; Jurisdiction. This Agreement shall be governed and construed in accordance with the laws of the State of Delaware excluding any choice of law rules which may direct the application of the law of another state.

12.8 Severability. If any provision of this Agreement is held to be illegal, invalid or unenforceable under any present or future law, and if the rights or obligations of any Party hereto under this Agreement will not be materially and adversely affected thereby, (a) such provision will be fully severable, (b) this Agreement will be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement will remain in full force and effect and will not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there will be added automatically as a part of this Agreement a legal, valid and enforceable provision as similar to the terms to such illegal, invalid or unenforceable provision as may be possible and reasonably acceptable to the Parties herein.

12.9 No Third Party Rights. Except as otherwise expressly set forth herein, no provision of this Agreement shall be deemed or construed in any way to result in the creation of any rights or obligations in any person not a Party to this Agreement.

12.10 Exhibits. The Exhibits referenced in this Agreement are an integral part of this Agreement and are incorporated herein by reference.

12.11 Force Majeure. If either Party is prevented from complying, either totally or in part, with any of the terms or provisions set forth herein by reason of force majeure, including, by way of example and not of limitation, fire, flood, explosion, storm, hurricane, strike, lockout or other labor dispute, riot, war, rebellion, accidents, acts of God, or acts of governmental agencies or instrumentalities, in each case to the extent beyond its control despite its commercially reasonable

efforts to avoid, minimize, and resolve such cause as promptly as possible, said Party shall (a) provide written notice of same to the other Party, and (b) subject to the obligations set forth above with respect to said Party's efforts to remove the disability, its obligations that are prevented from compliance by such force majeure are suspended, without liability, during such period of force majeure. Said notice shall be provided within ten (10) business days of the occurrence of such event and shall identify the requirements of this Agreement or such of its obligations as may be affected. The Party so affected shall give to the other Party a good faith estimate of the continuing effect of the force majeure condition and the duration of the affected Party's nonperformance.

12.12 No Other Relationship. It is expressly agreed that AAIPharma, on the one hand, and Company, on the other hand, shall be independent contractors and that nothing contained herein shall be deemed to create any joint venture or partnership between the Parties hereto, and, except as is expressly set forth herein, neither Party shall have any right by virtue of this Agreement to bind the other Party in any manner whatsoever.

12.13 Additional Product. The Parties covenant and agree that additional products may be added to this Agreement and such additional products shall be governed by the general conditions hereof with any special terms (including, without limitation, price) governed by an addendum hereto.

12.14 Dispute Resolution.

(a) Negotiated Settlement. In the event of a dispute regarding payment or the performance of Services pursuant to this Agreement (each, a "Dispute"), the Parties shall endeavor to negotiate in good faith an agreeable solution. If after [**] business days following receipt of a Party's written notification of a Dispute such Dispute has not been resolved, the Dispute shall be brought to the attention of the senior management of each Party and such senior manager or his/her designee will negotiate in good faith to define and implement a final resolution. The intent of this Section 12.14 is to encourage the Parties to work together to resolve any Dispute without having to rely on arbitration or any other legal proceeding. However, nothing in this Section 12.14 shall prevent or inhibit either Party to institute any other action to resolve such Dispute(s).

(b) Binding Arbitration. If not resolved in accordance with the preceding paragraph (a) then any controversy or claim arising out of or relating to this Agreement, or the breach thereof, shall be settled by arbitration administered by the American Arbitration Association in accordance with its Commercial Arbitration Rules, and judgment on the award rendered by the arbitrator(s) may be entered in any court having jurisdiction thereof.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties hereto have executed this Agreement as of the date first above written.

Marathon Pharmaceuticals, LLC

By: /s/ Patrick J. Morris

Printed Name: Patrick J. Morris

Title: EVP of Legal Affairs and General Counsel

Date: 10/9/15

AAIPharma Services Corp.

By: /s/ Syed T. Husain

Printed Name: Syed T. Husain

Title: Chief Commercial Officer

Date: 10/7/15

Exhibit A

Product(s), Batch Sizes, and Cost

Deflazacort tablets in the following strengths to be marketed in the Territory.

Dosage Strength	Dosage Form	Lot Size	Bottle Count	Price/ Bottle
6-mg	**Tablet**	**[**]**	**100**	**$[**]**
18-mg	**Tablet**	**[**]**	**30**	**$[**]**
30-mg	**Tablet**	**[**]**	**30**	**$[**]**
36-mg	**Tablet**	**[**]**	**30**	**$[**]**

Exhibit B

Specifications

[Attached]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 12 pages were omitted. [**]

Amendment #1

THIS AMENDMENT #1 ("Amendment") is entered into as of September 18, 2016 (the "Amendment Effective Date") by and between Alcami Corporation, formerly known as AAIPharma Services Corp. ("Alcami") and Marathon Pharmaceuticals, LLC ("Company").

WHEREAS, Company and Alcami entered into a Commercial Manufacturing Agreement with an effective date of September 18, 2015 (the "Agreement");

WHEREAS, the Parties have requested modifications to Exhibits A and B of the Agreement; and

WHEREAS, the Parties wishes to implement the requested modifications upon the terms and conditions set forth herein.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged by each party hereto to the other, both parties mutually agree as follows:

1. Capitalized terms not otherwise defined herein will have the meaning given to them in the Agreement.
2. Where found in the Agreement, references to AAIPharma Services Corp. and AAIPharma shall be deleted and replaced with Alcami Corporation and Alcami, respectively.
3. Exhibit A, Product(s), Batch Sizes, and Cost, shall be deleted in its entirety and replaced with the attached Exhibit A1.
4. The specifications in Exhibit B shall be deleted in their entirety and replaced with the attached Exhibit B1.
5. [**].
6. Notwithstanding, the Parties expressly agree that purchase orders #2348, #2349, #2350, #2351, #2365, and #2366 submitted by Company to Alcami prior to the Amendment Effective Date, shall be prepared and shipped according to the terms outlined in the previously agreed to Exhibit A and Exhibit B respectively.
7. [**].

Except as otherwise modified herein, the Agreement will remain in full force and effect.

ACKNOWLEDGED, ACCEPTED, AND AGREED TO:

Alcami Corporation	**Marathon Pharmaceuticals, LLC**
By: /s/ Syed T. Husain	By: /s/ Patrick J. Morris
Name: Syed T. Husain	Name: Patrick J. Morris
Title: Chief Commercial Officer	Title: EVP of Legal Affairs and General Counsel
Date: 11/30/2016	Date: 11/11/16

Exhibit A1

Product(s), Batch Sizes, and Cost

Deflazacort tablets in the following strengths to be marketed in the Territory:

Dosage Strength	Dosage Form	Lot Size (Bottles)	Bottle Count	Price/Bottle (USD)
3 mg	Tablet	[**]	100	$[**]
6 mg	Tablet	[**]	100	$[**]
18 mg	Tablet	[**]	30	$[**]
30 mg	Tablet	[**]	30	$[**]
36 mg	Tablet	[**]	30	$[**]

Secondary Packaging of Pre-Filled Deflazacort Oral Suspension Bottles
(13ml fill, 22.75 mg/ml)

Dosage Strength	Dosage Form	Lot Size (Bottles)	Fill Volume	Price/Bottle (USD)
22.75 mg/mL	Oral Suspension	[**]	13mL	$[**]

Exhibit B1

Specifications

[Attached]

[Note: not attached by the parties]

Amendment #2

THIS AMENDMENT #2 ("Amendment") is entered into as of January 6, 2017 (the "Amendment Effective Date") by and between Alcami Corporation, formerly known as AAIPharma Services Corp. ("Alcami") and Marathon Pharmaceuticals, LLC ("Company").

WHEREAS, Company and Alcami entered into a Commercial Manufacturing Agreement with an effective date of September 18, 2015 and as amended September 18, 2016 (the "Agreement");

WHEREAS, the Parties have requested modifications to the Agreement; and

WHEREAS, the Parties wish to implement the requested modifications upon the terms and conditions set forth herein.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is herby acknowledged by each party hereto to the other, both parties mutually agree as follows:

1. Capitalized terms not otherwise defined herein will have the meaning given to them in the Agreement.
2. Section 1.35, Territory, shall be deleted and replaced with the following:

"**1.35 "Territory"** shall mean the United States, its territories and possessions[**]."

Except as otherwise modified herein, the Agreement will remain in full force and effect.

IN WITNESS WHEREOF, the Parties hereto have executed this Amendment as of the Amendment Effective Date.

Alcami Corporation	**Marathon Pharmaceuticals, LLC**
By: /s/ Syed T. Husain	By: /s/ Patrick J. Morris
Name: Syed T. Husain	Name: Patrick J. Morris
Title: Chief Commercial Officer	Title: EVP of Legal Affairs and General Counsel
Date: 1/11/2017	Date: 1/6/17

EMPLOYMENT AGREEMENT

This EMPLOYMENT AGREEMENT (the "Agreement") is made as of July 8, 2014 (the "Effective Date"), by and between PTC Therapeutics, Inc., a Delaware corporation (the "Company") and Marcio Silva de Souza ("Executive"). In consideration of the mutual covenants contained in this Agreement, the Company and Executive agree as follows:

1. Employment. The Company agrees to continue to employ Executive and Executive agrees to continue to be employed by the Company on the terms and conditions set forth in this Agreement.

(a) Capacity. Executive shall serve the Company as Vice President, Global Marketing reporting to Mark Rothera, Chief Commercial Officer, or such senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Vice President, Global Marketing. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his/her position as Vice President, Global Marketing as may be reasonably assigned to Executive from time to time by the individual to whom s/he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080, subject to relocation and Section 2(c)(i) of this Agreement.

(b) Devotion of Duties; Representations. During the Term (as defined below) of Executive's employment with the Company, Executive shall devote his/her best efforts and full business time and energies to the business and affairs of the Company, and shall endeavor to perform the duties and services contemplated hereunder to the reasonable satisfaction of the individual to whom s/he reports and the Board. During the Term of Executive's employment with the Company, Executive shall not, without the prior written approval of the Company (by action of the Board), undertake any other employment from any person or entity or serve as a director of any other company; provided, however, that (i) the Company will entertain requests as to such other employment or directorships in good faith and (ii) Executive will be eligible to participate in any policy relating to outside activities that is applicable to the senior executives of the Company and approved by the Board after the date hereof.

2. Term of Employment.

(a) Executive's employment hereunder shall commence on the Effective Date. Executive's employment hereunder shall be terminated upon the first to occur of the following:

(i) Immediately upon Executive's death;

(ii) By the Company:

(A) By written notice to Executive effective the date of such notice, following the Disability of Executive. "Disability" means that Executive (i) is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to

result in death or can be expected to last for a continuous period of not less than 12 months, or (ii) is, by reason of any medically determinable physical or mental impairment which can be expected to last for a continuous period of not less than 12 months, receiving income replacement benefits for a period of not less than three months under an accident and health plan covering employees of the Company. Such incapacity shall be determined by a physician chosen by the Company and reasonably satisfactory to Executive (or Executive's legal representative) upon examination requested by the Company (to which Executive hereby agrees to submit). Notwithstanding the foregoing, such Disability must result in Executive becoming "Disabled" within the meaning of Section 409A(a)(2)(C) of the Internal Revenue Code of 1986, as amended (the "Code") and the guidance issued thereunder. (In this Agreement we refer to Section 409A of the Code and any guidance issued thereunder as "Section 409A").

(B) By written notice to Executive, effective the date of such notice, for Cause (as defined below); or

(C) By written notice to Executive, effective ninety (90) days after the date of such notice and subject to Section 4 hereof, without Cause; or

(iii) By Executive:

(A) At any time by written notice to the Company, effective forty-five (45) days after the date of such notice; or

(B) By written notice to the Company for Good Reason (as defined below), effective on the date specified in such notice.

The term of Executive's employment by the Company under this Agreement is referred to herein as the "Term."

(b) Definition of "Cause". For purposes of this Agreement, "Cause" shall, pursuant to the reasonable good faith determination by a majority of the Board (excluding Executive) as documented in writing, include: (i) the willful and continued failure by Executive to substantially perform Executive's material duties or responsibilities under this Agreement (other than such a failure as a result of Disability); (ii) any action or omission by Executive involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) Executive's conviction of a felony, either in connection with the performance of Executive's obligations to the Company or which otherwise shall adversely affect Executive's ability to perform such obligations or shall materially adversely affect the business activities, reputation, goodwill or image of the Company; (iv) the material breach of a fiduciary duty to the Company; or (v) the material breach by Executive of any of the provisions of this Agreement, provided that any breach of Executive's obligations with respect to Sections 5 or 6 of this Agreement, subject to the cure provision in the next sentence, shall be deemed "material." In respect of the events described in clauses (i) and (v) above, the Company shall give Executive notice of the failure of performance or breach, reasonable as to time, place and manner in the circumstances, and a 30-day opportunity to cure, provided that such failure of

performance or breach is reasonably amenable to cure as determined by the Board in its sole discretion.

(c) Definition of "Good Reason". For purposes of this Agreement, a "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from Executive within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Executive has consented to the condition that would otherwise be a basis for Good Reason:

(i) A change in the principal location at which Executive provides services to the Company to a location more than fifty (50) miles from such principal location and/or to a location in New York City (either of which change, the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which Executive provides services to the Company), provided that such a relocation shall not be deemed to occur under circumstances where Executive's responsibilities require him/her to work at a location other than the corporate headquarters for a reasonable period of time;

(ii) A material adverse change by the Company in Executive's duties, authority or responsibilities as Vice President, Global Marketing of the Company which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business.

(iii) A material reduction in Executive's base compensation (including Base Salary) except if the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change;

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by Executive; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

(d) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company);

(iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the Securities Exchange Act of 1934 (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) Executive forms part of an executive management team that consists of substantially the same group of individuals and Executive is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

3. Compensation.

(a) Base Salary. Executive's minimum base salary during the Term shall be at the rate of $280,000 per year (the "Base Salary"). Base Salary shall be payable in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, less any amounts required to be withheld under applicable law. The Base Salary will be subject to adjustment from time to time in the sole discretion of the Board; provided that, the Company covenants that it shall not reduce the Base Salary below $280,000 or the Base Salary then in effect immediately prior to the reduction unless (i) Executive consents to such reduction, or (ii) the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change.

(b) Bonus. In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 30% of Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned. For the 2014 calendar year only, Executive will be eligible to receive a minimum annual bonus at 30% target of $280,000 (which represents Executive's Base Salary for an entire calendar year, even though he was employed for less than the full 2014 calendar year).

(c) Equity Compensation. Executive acknowledges receipt of an inducement grant of 30,000 options to purchase shares of common stock of PTC (the "Inducement Grant"). Such award was granted pursuant to the inducement grant exception under NASDAQ rules and was intended to serve as a material inducement to Executive entering into employment with PTC. For clarity, the Inducement Grant was in lieu of any grant under the Company's equity and long term incentive plan(s) for the 2014 calendar year. Commencing in 2015, Executive shall be eligible to participate in PTC's annual equity and long term incentive plan(s) and may be eligible to receive discretionary awards under such plan(s), subject to the terms and conditions of such plan(s). Except as explicitly set forth below, Executive's rights with respect to equity (including stock options) shall be covered in PTC's equity and long term incentive plan(s) and separate stock option certificates or agreements for each grant.

(i) Accelerated Vesting.

(A) For the avoidance of doubt, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason, neither the unvested portion of the Inducement Grant nor any unvested equity awards granted under the Company's equity and long-term incentive plan(s) shall be subject to any accelerated vesting except as otherwise provided for in the applicable award agreement or in Section 3(c)(i)(B) below.

(B) Except as otherwise provided in the applicable award, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs during the Term (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of the unvested portion of the Inducement Grant and all of Executive's outstanding unvested equity awards granted under the Company's equity and long-term incentive plan(s) shall vest immediately.

(d) Vacation. Executive is eligible for time off programs outlined in the Company's Time Off Policy. Executive shall accrue over the calendar year 160 hours of paid vacation. Executive may accrue up to 200 hours of vacation. Once Executive has reached the maximum accrual, no further vacation time will be accrued unless and until the Executive uses vacation time. Upon termination of employment, the value of Executive's current balance of accrued but unused vacation shall be paid out in cash based on his/her Base Salary that was in effect immediately prior to his/her termination of employment.

(e) Fringe Benefits. Executive shall be entitled to participate in any employee benefit plans that the Company makes available to its senior executives (including, without limitation, group life, disability, medical, dental and other insurance, retirement, pension, profit-sharing and similar plans) (collectively, the "Fringe Benefits"), provided that the Fringe Benefits shall not include any stock option or similar plans relating to the grant of equity securities of the Company. These benefits may be modified or changed from time to time at the sole discretion of

the Company. Where a particular benefit is subject to a formal plan (for example, medical or life insurance), eligibility to participate in and receive any particular benefit is governed solely by the applicable plan document, and eligibility to participate in such plan(s) may be dependent upon, among other things, a physical examination.

(f) Reimbursement of Expenses. Executive shall be entitled to reimbursement for all ordinary and reasonable out-of-pocket business expenses that are reasonably incurred by him/her in furtherance of the Company's business in accordance with reasonable policies adopted from time to time by the Company for senior executives.

(g) Sign-On Bonus. Executive acknowledges receipt of a one-time, lump sum bonus of $20,000. Executive further acknowledges and agrees that he shall be required to refund to PTC the full gross amount of such bonus in the event that, prior to the first anniversary of Executive's Start Date, Executive resigns his employment for any reason or PTC terminates Executive's employment for Cause.

(h) Taxes and Withholdings. The Company shall deduct and withhold from all compensation and benefits under this Agreement all social security and other federal, state and local taxes and charges which currently are or which hereafter may be required by law to be so deducted and withheld.

4. Severance Compensation.

(a) In the event of any termination of Executive's employment for any reason the Company shall pay Executive (or Executive's estate) such portions of Executive's Base Salary as have accrued prior to such termination and have not yet been paid, together with (i) amounts for accrued unused vacation days (as provided above), (ii) any amounts for expense reimbursement which have been properly incurred or the Company has become obligated to pay prior to termination and have not been paid as of the date of such termination and (iii) the amount of any Bonus previously granted to Executive by the Board but not yet paid, which amount shall not include any pro rata portion of any Bonus which would have been earned if such termination had not occurred (the "Accrued Obligations"). Such amounts shall be paid as soon as possible after termination.

(b) In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth below for six (6) months following Executive's termination of employment (the "Severance Period"). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution (and, as applicable, nonrevocation) of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his/her Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing no later than sixty (60) days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his/her Base Salary for the Severance Period, which total amount shall be payable in a lump sum no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) The Company shall continue to provide Executive and his/her then-enrolled eligible dependents with group health insurance and shall continue to pay the amount of the premium as in effect on the date of such termination for the Severance Period commencing on the effective date of such termination, subject to applicable law and the terms of the respective policies; provided that the Company's obligation to provide the benefits contemplated herein shall terminate upon Executive's becoming eligible for coverage under the medical benefits program of a subsequent employer. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.

(iii) Compliance with Section 409A. Subject to the provisions in this Section 4(b)(iii), any severance payments or benefits under this Agreement shall begin only upon the date of Executive's "separation from service" (determined as set forth below) which occurs on or after the date of termination of Executive's employment. The following rules shall apply with respect to the distribution of the severance payments and benefits, if any, to be provided to Executive under this Agreement:

(1) It is intended that each installment of the severance payments and benefits provided under this Agreement shall be treated as a separate "payment" for purposes of Section 409A. Neither the Company nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(2) If, as of the date of Executive's "separation from service" from the Company, Executive is not a "specified employee" (within the meaning of Section 409A), then each installment of the severance payments and benefits shall be made on the dates and terms set forth in this Agreement.

(3) If, as of the date of Executive's "separation from service" from the Company, Executive is a "specified employee" (within the meaning of Section 409A), then:

(A) Each installment of the severance payments and benefits due under this Agreement that, in accordance with the dates and terms set forth herein, will in all circumstances, regardless of when the separation from service occurs, be paid within the short-term deferral period (as defined under Section 409A) shall be treated as a short-term deferral within the meaning of Treasury Regulation Section 1.409A-1(b)(4) to the maximum extent permissible under Section 409A and such payments and benefits shall be paid or provided on the dates and terms set forth in this Agreement; and

(B) Each installment of the severance payments and benefits due this Agreement that is not described in Section 4(b)(iii)(3)(A) above and that would, absent this subsection, be paid within the six-month period following Executive's "separation from service" from the Company shall not be paid until the date that is six months and one day after such separation from service (or, if earlier, Executive's death), with any such installments that are required to be delayed being accumulated during the six-month period and paid in a lump sum on the date that is six months and one day following Executive's separation from service and any subsequent installments, if any, being paid in accordance with the dates and terms set forth herein; provided, however, that the preceding provisions of this sentence shall not apply to any installment of severance payments and benefits if and to the maximum extent that such installment is deemed to be paid under a separation pay plan that does not provide for a deferral of compensation by reason of the application of Treasury Regulation 1.409A-1(b)(9)(iii) (relating to separation pay upon an involuntary separation from service). Any installments that qualify for the exception under Treasury Regulation Section 1.409A-1(b)(9)(iii) must be paid no later than the last day of Executive's second taxable year following the taxable year in which the separation from service occurs.

(4) The determination of whether and when Executive's separation from service from the Company has occurred shall be made in a manner consistent with, and based on the presumptions set forth in, Treasury Regulation Section 1.409A-1(h). Solely for purposes of this Section 4(b)(iii), "Company" shall include all persons with whom the Company would be considered a single employer under Section 414(b) and 414(c) of the Code.

(5) All reimbursements and in-kind benefits provided under this Agreement shall be made or provided in accordance with the requirements of Sections 409A to the extent that such reimbursements or in-kind benefits are subject to Section 409A, including, where applicable, the requirements that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement), (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses eligible for reimbursement in any other calendar year, (iii) the reimbursement of an eligible expense will be made on or before the last day of the calendar year following the year in which the expense is incurred and (iv) the right to

reimbursement is not subject to set off or liquidation or exchange for any other benefit.

(6) Notwithstanding anything herein to the contrary, the Company shall have no liability to Executive or to any other person if the payments and benefits provided hereunder that are intended to be exempt from or compliant with Section 409A are not so exempt or compliant.

(c) In the event that Executive's employment hereunder is terminated (i) by Executive for other than a Good Reason, or (ii) by the Company for Cause, or (iii) as a result of Executive's death or Disability, then the Company will pay to Executive the Accrued Obligations. The Company shall have no obligation to pay Executive (or Executive's estate) any other compensation following such termination except as provided in Section 4(a).

(d) Modified Section 280G Cutback.

(i) Notwithstanding any other provision of this Agreement, except as set forth in Section 4(d)(ii), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide to Executive a portion of any "Contingent Compensation Payments" (as defined below) that Executive would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for Executive. For purposes of this Section 4(d), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(ii) Notwithstanding the provisions of Section 4(d)(i), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by Executive if the Eliminated Payments (determined without regard to this sentence) were paid to him/her (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of Executive's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 4(d)(ii) shall be referred to as a "Section 4(d)(ii) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(iii) For purposes of this Section 4(d) the following terms shall have the following respective meanings:

(1) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(2) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(iv) Any payments or other benefits otherwise due to Executive following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 4(d)(iv). Within 30 days after each date on which Executive first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify Executive (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 4(d)(ii) Override is applicable. Within 30 days after delivery of such notice to Executive, Executive shall deliver a response to the Company (the "Executive Response") stating either (A) that s/he agrees with the Company's determination pursuant to the preceding sentence or (B) that s/he disagrees with such determination, in which case s/he shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 4(d)(ii) Override is applicable. In the event that Executive fails to deliver an Executive Response on or before the required date, the Company's initial determination shall be final. If Executive states in the Executive Response that s/he agrees with the Company's determination, the Company shall make the Potential Payments to Executive within three business days following delivery to the Company of the Executive Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If Executive states in the Executive Response that s/he disagrees with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, Executive and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in South Plainfield, New Jersey, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to Executive those Potential Payments as to which there is no dispute between the Company and Executive regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(v) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by Executive for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by Executive in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(vi)The provisions of this Section 4(d) are intended to apply to any and all payments or benefits available to Executive under this Agreement or any other agreement or plan of the Company under which Executive receives Contingent Compensation Payments.

(vii) Notwithstanding Sections 4(d)(i)-(vi) hereof, until the closing of the first underwritten public offering of common stock of the Company, in the event that it shall be determined that any payment or benefit (including any accelerated vesting of options or other equity awards) made or provided, or to be made or provided, by the Company (or any successor thereto or affiliate thereof) to or for the benefit of Executive, whether pursuant to the terms of this Agreement, any other agreement, plan, program or arrangement of or with the Company (or any successor thereto or affiliate thereof) or otherwise, may be subject to the excise tax imposed by Section 4999 of the Code or any comparable tax imposed by any replacement or successor provision of United States tax law, then upon the request of Executive, the Company shall use reasonable efforts to procure a shareholder vote in satisfaction of the shareholder approval requirements described in Treas. Reg. Section 1.280G-1, Q&A-7.

5. Executive Covenants.

(a) Confidential Information. Executive recognizes and acknowledges the competitive and proprietary aspects of the business of the Company, and that as a result of Executive's employment, Executive recognizes and acknowledges that s/he will have access to,

and will continue to be involved in the development of, Confidential Information (as defined below) of the Company. As used herein, "Confidential Information" shall mean and include trade secrets, knowledge and other confidential information of the Company, which Executive has acquired, no matter from whom or on what matter such knowledge or information may have been acquired, heretofore or hereafter, concerning the content and details of the business of the Company, and which is not known to the general public, including but not limited to: (a) new products, product betterments and other inventions, formulas, processes, methods, materials, material combinations, manner of preparations, technical production procedures and information, alarm and security codes and procedures, sources of technology, and sources of supply of raw and finished materials and other products; (b) financial and accounting records; (c) the identity of employees, consultants, independent contractors, customers, business development partners, licensees, suppliers, creditors or other parties with which the Company has business dealings, the nature of the relationship with such persons, or any other information relating to such persons or the Company's dealings with such persons; and (d) computer software used by the Company or provided to the customers of the Company unless publicly available.

(i) For as long as Executive is employed and at all times thereafter, Executive shall not, directly or indirectly, communicate, disclose or divulge to any person or entity, or use for Executive's own benefit or the benefit of any person (other than the Company), any Confidential Information, except as permitted in subparagraph (iii) below. Upon termination of Executive's employment, or at any other time at the request of the Company, Executive agrees to deliver promptly to the Company all Confidential Information, including, but not limited to, customer and supplier lists, files and records, in Executive's possession or under Executive's control. Executive further agrees that s/he will not make or retain any copies of any of the foregoing and will so represent to the Company upon termination of Executive's employment.

(ii) Executive shall disclose immediately to the Company any trade secrets or other Confidential Information conceived or developed by Executive at any time during Executive's employment. Executive hereby assigns and agrees to assign to the Company Executive's entire right, title and interest in and to all Confidential Information. Such assignment shall include, without limitation, the rights to obtain patent or copyright protection, thereon in the United States and foreign countries. Executive agrees to provide all reasonable assistance to enable the Company to prepare and prosecute any application before any governmental agency for patent or copyright protection or any similar application with respect to any Confidential Information. Executive further agrees to execute all documents and assignments and to make all oaths necessary to vest ownership of such intellectual property rights in the Company, as the Company may request. These obligations shall apply whether or not the subject thereof was conceived or developed at the suggestion of the Company, and whether or not developed during regular hours of work or while on the premises of the Company.

(iii) Executive shall at all times, both during and after termination of this Agreement by either Executive or the Company, maintain in confidence and shall not, without prior written consent of the Company, use, except in the course of performance of Executive's duties for the Company or as required by legal process (provided that Executive will promptly notify the Company of such legal process except

with respect to any confidential government investigation), disclose or give to others any Confidential Information. In the event Executive is questioned by anyone not employed by the Company or by an employee of or a consultant to the Company not authorized to receive such information, in regard to any such information or any other secret or confidential work of the Company, or concerning any fact or circumstance relating thereto, Executive will promptly notify the Company.

(b) Non-Competition and Non-Solicitation. Executive recognizes that the Company is engaged in a competitive business and that the Company has a legitimate interest in protecting its trade secrets, confidential business information, and customer, business development partner, licensee, supplier, and credit and/or financial relationships. Accordingly, in exchange for valuable consideration, including without limitation Executive's access to confidential business information and continued at-will employment, Executive agrees that, during the Term hereof and for a period of eighteen (18) months thereafter, Executive shall not:

(i) directly or indirectly, whether for himself or for any other person or entity, and whether as a proprietor, principal, shareholder, partner, agent, employee, consultant, independent contractor, or in any other capacity whatsoever, undertake or have any interest in (other than the passive ownership of publicly registered securities representing an ownership interest of less than 1%), engage in or assume any role involving directly or indirectly the Company's Field of Interest (or any portion thereof) or any other business in which the Company is engaged and for which the employee has rendered services while employed by the Company, or enter into any agreement to do any of the foregoing; or

(ii) initiate contact with (including without limitation phone calls, press releases and the sending or delivering of announcements), or in any manner solicit, directly or indirectly, any customers, business development partners, licensors, licensees, or creditors (including institutional lenders, bonding companies and trade creditors) of the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to transfer any of their business with the Company to any person or entity other than the Company; or

(iii) initiate contact with, or in any manner solicit, directly or indirectly, any supplier of goods, services or materials to the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to supply the same or similar inventory, goods, services or materials (except generally available inventory, goods, services or materials) to any person or entity other than the Company; or

(iv) directly or indirectly recruit, solicit or otherwise induce or influence any employee or independent contractor of the Company to discontinue or modify his or her employment or engagement with the Company, or employ or contract with any such employee or contractor for the provision of services.

(c) Definition of "Field of Interest". The term Company's "Field of Interest" shall mean the research, development and commercialization of products and strategies relating to: (i) therapies for genetic disorders or diseases that include cystic fibrosis, Duchenne muscular

dystrophy, other diseases caused in whole or part by nonsense (or stop) codons, and other genetic diseases as to which the Company engages in the research, development or commercialization of drugs; anti-angiogenic therapies that target VEGF protein production for cancer; and antiviral therapies for the Hepatitis C virus (HCV); and (ii) other therapeutic targets, mechanisms of action and/or therapies in which the Company has a research, development or commercialization program.

(d) Definition of "Customer". The term "customer" or "customers" shall include any person or entity (a) that is a current customer of the Company, (b) that was a customer of the Company at any time during the preceding twenty-four (24) months or (c) to which the Company made a written presentation for the solicitation of business at any time during the preceding twenty-four (24) months.

(e) Reasonableness of Restrictions. Executive further recognizes and acknowledges that (i) the types of employment which are prohibited by this Section 5 are narrow and reasonable in relation to the skills which represent Executive's principal salable asset both to the Company and to Executive's other prospective employers, and (ii) the broad geographical scope of the provisions of this Section 5 is reasonable, legitimate and fair to Executive in light of the global nature of the Company's business, particularly pharmaceutical research and development, and in light of the limited restrictions on the type of employment prohibited herein compared to the types of employment for which Executive is qualified to earn Executive's livelihood.

(f) Remedies. Executive acknowledges that a breach of this Section 5 will cause great and irreparable injury and damage, which cannot be reasonably or adequately compensated by money damages. Accordingly, Executive acknowledges that the remedies of injunction and specific performance shall be available in the event of such a breach, in addition to money damages, costs and attorneys' fees, and other legal or equitable remedies, and that the Company shall be entitled as a matter of course to an injunction pending trial, without the posting of bond or other security. Any period of restriction set forth in this Section 5 shall be extended for a period of time equal to the duration of any breach or violation hereof.

(g) Notification. Any person employing Executive or evidencing any intention to employ Executive may be notified as to the existence and provisions of this Agreement.

(h) Modification of Covenants; Enforceability. In the event that any provision of this Section 5 is held to be in any respect an unreasonable restriction, then the court so holding may modify the terms thereof, including the period of time during which it operates or the geographic area to which it applies, or effect any other change to the extent necessary to render this section enforceable, it being acknowledged by the parties that the representations and covenants set forth herein are of the essence of this Agreement.

(i) Subsidiaries. For purposes of Sections 5 and 6 of this Agreement, "Company" shall include all direct and indirect subsidiaries of the Company. An entity shall be deemed to be a subsidiary of the Company if the Company directly or indirectly owns or controls 50% or more of the equity interest in such entity.

6. Ownership of Ideas, Copyrights and Patents.

(a) Property of the Company. Executive agrees that all ideas, discoveries, creations, manuscripts and properties, innovations, improvements, know-how, inventions, designs, developments, apparatus, techniques, methods, biological processes, cell lines, laboratory notebooks and formulae, whether patentable, copyrightable or not, which Executive may conceive, reduce to practice or develop, alone or in conjunction with another, or others, whether during or out of regular business hours, and whether at the request or upon the suggestion of the Company, or otherwise, in the course of performing services for the Company in any capacity, whether heretofore or hereafter, (collectively, "the Inventions") are and shall be the sole and exclusive property of the Company, and that Executive shall not publish any of the Inventions without the prior written consent of the Company. Executive hereby assigns to the Company all of Executive's right, title and interest in and to all of the foregoing. Executive further represents and agrees that to the best of Executive's knowledge and belief none of the Inventions will violate or infringe upon any right, patent, copyright, trademark or right of privacy, or constitute libel or slander against or violate any other rights of any person, firm or corporation and that Executive will use his/her best efforts to prevent any such violation.

(b) Cooperation. At any time during or after the Term, Executive agrees that s/he will fully cooperate with the Company, its attorneys and agents in the preparation and filing of all papers and other documents as may be required to perfect the Company's rights in and to any of such Inventions, including, but not limited to, executing any lawful document (including, but not limited to, applications, assignments, oaths, declarations and affidavits) and joining in any proceeding to obtain letters patent, copyrights, trademarks or other legal rights of the United States and of any and all other countries on such Inventions, provided that any patent or other legal right so issued to Executive, personally, shall be assigned by Executive to the Company without charge by Executive. Executive further designates the Company as his/her agent for, and grants to the Company a power of attorney with full power of substitution, which power of attorney shall be deemed coupled with an interest, for the purpose of effecting the foregoing assignments from Executive to the Company. Company will bear the reasonable expenses which it causes to be incurred in Executive's assisting and cooperating hereunder. Executive waives all claims to moral rights in any Inventions.

7. Disclosure to Future Employers. The Company may provide in its discretion, a copy of the covenants contained in Sections 5 and 6 of this Agreement to any business or enterprise which Executive may directly, or indirectly, own, manage, operate, finance, join, control or in which Executive participates in the ownership, management, operation, financing, or control, or with which Executive may be connected as an officer, director, employee, partner, principal, agent, representative, consultant or otherwise.

8. Records. Upon termination of Executive's relationship with the Company, Executive shall deliver to the Company any property of the Company which may be in

Executive's possession including products, materials, memoranda, notes, records, reports, or other documents or photocopies of the same.

9. Insurance. The Company, in its sole discretion, may apply for and procure in its own name (whether or not for its own benefit) policies of insurance insuring Executive's life. Executive agrees to submit to reasonable medical or other examinations and to execute and deliver any applications or other instruments in writing that are reasonably necessary to effectuate such insurance. No adverse employment actions may be based upon the results of any such exam or the failure by the Company to obtain such insurance.

10. No Conflicting Agreements. Executive hereby represents and warrants that Executive has no commitments or obligations inconsistent with this Agreement.

11. "Market Stand-Off" Agreement. Executive agrees, if requested by the Company and an underwriter of common stock (or other securities) of the Company, not to sell or otherwise transfer or dispose of any common stock (or other securities) of the Company held by Executive during a period not to exceed one hundred and eighty (180) days following the effective date of the first underwritten public offering of common stock of the Company, offered on a firm commitment basis pursuant to a registration statement filed with the Securities and Exchange Commission (or any successor agency of the Federal government administrating the Securities Act of 1933, as amend, and the Securities Exchange Act of 1934, as amended) under the Securities Act of 1933, as amended, on Form S-1 or its then equivalent, and to enter into an agreement to such effect. The Company may impose stop-transfer instructions with respect to the shares (or securities) subject to the foregoing restriction until the end of said period.

12. General.

(a) Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address as follows:

If to the Company: PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080
USA
Attention: Legal Department
Telephone: (908) 222-7000

With an email copy to: legal@ptcbio.com

If to Executive: Marcio Silva de Souza
7 Lenore Road
Califon, NJ 07830

or to such other address as a party may designate by notice hereunder, and shall be either (i) delivered by hand, (ii) sent by overnight courier, or (iii) sent by registered or certified mail, return receipt requested, postage prepaid. All notices, requests, consents and other communications hereunder shall be deemed to have been given either (i) if by hand, at the time of the delivery thereof to the receiving party at the address of such party set forth above, (ii) if

sent by overnight courier, on the next business day following the day such notice is delivered to the courier service, or (iii) if sent by registered or certified mail, on the fifth (5th) business day following the day such mailing is made.

(b) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, except with respect to the equity and fringe benefit arrangements referred to in Subsections 3(c) and (e) above. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(c) Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(d) Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(e) Assignment. The Company shall assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of the Company's business or that aspect of the Company's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of the Company.

(f) Benefit. All statements, representations, warranties, covenants and agreements in this Agreement shall be binding on the parties hereto and shall inure to the benefit of the respective successors and permitted assigns of each party hereto. Nothing in this Agreement shall be construed to create any rights or obligations except among the parties hereto, and no person or entity shall be regarded as a third-party beneficiary of this Agreement.

(g) Governing Law. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the law of The State of New Jersey, without giving effect to the conflict of law principles thereof.

(h) Jurisdiction and Service of Process. Any legal action or proceeding with respect to this Agreement shall be brought in the courts of The State of New Jersey or of the United States of America for the District of New Jersey. By execution and delivery of this Agreement, each of the parties hereto accepts for itself and in respect of its property, generally and unconditionally, the jurisdiction of the aforesaid courts. Each of the parties hereto irrevocably consents to the service of process of any of the aforementioned courts in any such action or proceeding by the mailing of copies thereof by certified mail, postage prepaid, to the party at its address set forth in Section 12(a) hereof.

(i) Severability. The parties intend this Agreement to be enforced as written. However, (i) if any portion or provision of this Agreement shall to any extent be declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby or otherwise, the Company and Executive agrees that the court making such determination shall have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases ("blue-penciling"), and in its reduced or blue-penciled form such provision shall then be enforceable and shall be enforced.

(j) Headings and Captions; Interpretation. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify, or affect the meaning or construction of any of the terms or provisions hereof. The provisions of the following Sections of this Agreement are in addition to, and do not limit, each other: Sections 6 and 5(a); Sections 7 and 5(g); Sections 12(k) and 12(f); and Sections 12(l) and 12(d).

(k) Injunctive Relief. Executive hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Section 5 or 6 of this Agreement will result in substantial, continuing and irreparable injury to the Company. Therefore, Executive hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction.

(l) No Waiver of Rights, Powers and Remedies. No failure or delay by a party hereto in exercising any right, power or remedy under this Agreement, and no course of dealing between the parties hereto, shall operate as a waiver of any such right, power or remedy of the party. No single or partial exercise of any right, power or remedy under this Agreement by a party hereto, nor any abandonment or discontinuance of steps to enforce any such right, power or remedy, shall preclude such party from any other or further exercise thereof or the exercise of any other right, power or remedy hereunder. The election of any remedy by a party hereto shall not constitute a waiver of the right of such party to pursue other available remedies. No notice to or demand on a party not expressly required under this Agreement shall entitle the party receiving such notice or demand to any other or further notice or demand in similar or other circumstances or constitute a waiver of the rights of the party giving such notice or demand to any other or further action in any circumstances without such notice or demand.

(m) Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

(n) Survival. The provisions of Sections 4, 5, 6, 7, 8, 11 and 12 shall survive the termination of this Agreement and Executive's employment hereunder in accordance with their terms.

(o) WAIVER OF TRIAL BY JURY. THE PARTIES IRREVOCABLY WAIVE THEIR RESPECTIVE RIGHT TO A TRIAL BY JURY REGARDING ANY DISPUTE, CLAIM OR CAUSE OF ACTION ARISING OUT OF, CONCERNING, OR RELATED TO EXECUTIVE'S EMPLOYMENT WITH THE COMPANY OR THIS AGREEMENT.

(p) Knowing and Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement knowingly and voluntarily and without any duress or undue influence by PTC or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and fully understands it, including that Executive is waiving the right to a jury trial. Executive further agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

IN WITNESS THEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

/s/ Mark E. Boulding
Name: Mark E. Boulding
Title: Executive Vice President and Chief Legal Officer

Agreed and Accepted

/s/ Marcio Silva de Souza
Name: Marcio Silva de Souza

EXHIBIT A

Sample Separation and Release Agreement

[Insert Date]

[Insert Employee Name]
[Insert Employee Address]

Dear **[Insert Employee Name]**:

In connection with the termination of your employment with PTC Therapeutics, Inc. (the "Company") on **[Termination Date]**, you are eligible to receive the Severance Compensation as described in Section 4 of the Employment Agreement executed between you and the Company on [**Insert Date**] (the "Employment Agreement") if you sign and return this letter agreement to me by **[Return Date – e.g., 21 days from date of receipt of this letter agreement]** and it becomes binding between you and the Company. By signing and returning this letter agreement [and not revoking your acceptance], you will be agreeing to the terms and conditions set forth in the numbered paragraphs below, including the release of claims set forth in paragraph 3. Therefore, you are advised to consult with an attorney before signing this letter agreement and you may take up to [twenty-one (21) days] to do so. [If you sign this letter agreement, you may change your mind and revoke your agreement during the seven (7) day period after you have signed it by notifying me in writing. If you do not so revoke, this letter agreement will become a binding agreement between you and the Company upon the expiration of the seven (7) day period.]

If you choose not to sign and return this letter agreement by **[Return Date-Same as Above][**, or if you timely revoke your acceptance in writing], you shall not receive any Severance Compensation from the Company. You will, however, receive payment for your final wages and any unused vacation time accrued through the Termination Date, as defined below, on the Company's regular payroll date immediately following the Termination Date. Also, regardless of signing this letter agreement, you may elect to continue receiving group medical insurance pursuant to the federal "COBRA" law, 29 U.S.C. § 1161 et seq. If you so elect, you shall pay all premium costs on a monthly basis for as long as, and to the extent that, you remain eligible for COBRA continuation. You should consult the COBRA materials to be provided by the Company for details regarding these benefits. All other benefits will cease upon your Termination Date in accordance with the plan documents.

The following numbered paragraphs set forth the terms and conditions that will apply if you timely sign and return this letter agreement and do not revoke it in writing within the seven (7) day period.

1. **Termination Date** – Your effective date of termination from the Company is **[Insert Date]** (the "Termination Date").

2. **Release** – In consideration of the payment of the Severance Compensation, which you acknowledge you would not otherwise be entitled to receive, you hereby fully, forever,

irrevocably and unconditionally release, remise and discharge the Company, its affiliates, subsidiaries, parent companies, predecessors, and successors, and all of their respective past and present officers, directors, stockholders, partners, members, employees, agents, representatives, plan administrators, attorneys, insurers and fiduciaries (each in their individual and corporate capacities) (collectively, the "Released Parties") from any and all claims, charges, complaints, demands, actions, causes of action, suits, rights, debts, sums of money, costs, accounts, reckonings, covenants, contracts, agreements, promises, doings, omissions, damages, executions, obligations, liabilities, and expenses (including attorneys' fees and costs), of every kind and nature that you ever had or now have against any or all of the Released Parties, including, but not limited to, any and all claims arising out of or relating to your employment with and/or separation from the Company, including, but not limited to, all claims under Title VII of the Civil Rights Act of 1964, 42 U.S.C. § 2000e et seq., the Americans With Disabilities Act of 1990, 42 U.S.C. § 12101 et seq., the Age Discrimination in Employment Act, 29 U.S.C. § 621 et seq., the Genetic Information Nondiscrimination Act of 2008, 42 U.S.C. § 2000ff et seq., the Family and Medical Leave Act, 29 U.S.C. § 2601 et seq., the Worker Adjustment and Retraining Notification Act ("WARN"), 29 U.S.C. § 2101 et seq., the Rehabilitation Act of 1973, 29 U.S.C. § 701 et seq., Executive Order 11246, Executive Order 11141, the Fair Credit Reporting Act, 15 U.S.C. § 1681 et seq., and the Employee Retirement Income Security Act of 1974 ("ERISA"), 29 U.S.C. § 1001 et seq., all as amended; all claims arising out of the New Jersey Law Against Discrimination, N.J. Stat. Ann. § 10:5-1 et seq., the New Jersey Family Leave Act, N.J. Stat. Ann. § 34:11B-1 et seq., the New Jersey Conscientious Employee Protection Act, N.J. Stat. Ann. § 34:19-1 et seq., and the N.J. Stat. Ann. § 34:11-56.1 et seq. (New Jersey equal pay law), all as amended; all common law claims including, but not limited to, actions in defamation, intentional infliction of emotional distress, misrepresentation, fraud, wrongful discharge, and breach of contract, including without limitation, all claims arising from the Employment Agreement; all state and federal whistleblower claims to the maximum extent permitted by law; all claims to any non-vested ownership interest in the Company, contractual or otherwise; and any claim or damage arising out of your employment with and/or separation from the Company (including a claim for retaliation) under any common law theory or any federal, state or local statute or ordinance not expressly referenced above; provided, however, that nothing in this letter agreement shall (i) prevent you from filing a charge with, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or a state fair employment practices agency (except that you acknowledge that you may not recover any monetary benefits in connection with any such claim, charge or proceeding) or (ii) deprive you of any rights you may have to be indemnified by the Company as provided in any agreement between the Company and you or pursuant to the Company's Certificate of Incorporation or by-laws.

3. **Non-Disclosure, Non-Competition and Non-Solicitation** – You acknowledge and reaffirm your obligation to keep confidential and not disclose all non-public information concerning the Company and its clients that you acquired during the course of your employment with the Company, as stated more fully in Section 5 of the Employment Agreement, which remains in full force and effect.

4. **Return of Company Property** – You confirm that you have returned to the Company all keys, files, records (and copies thereof), equipment (including, but not limited to, computer hardware, software and printers, wireless handheld devices, cellular phones, smartphones, tablets, etc.), Company identification, and any other Company-owned property in your possession or control and have left intact all electronic Company documents, including but not limited to those which you developed or helped to develop during your employment. You further confirm that you have cancelled all accounts for your benefit, if any, in the Company's name, including but not limited to, credit cards, telephone charge cards, cellular phone and/or wireless data accounts and computer accounts.

5. **Business Expenses and Final Compensation** – You acknowledge that you have been reimbursed by the Company for all business expenses incurred in conjunction with the performance of your employment and that no other reimbursements are owed to you. You further acknowledge that you have received payment in full for all services rendered in conjunction with your employment by the Company, including payment for all wages, bonuses and accrued, unused vacation time, and that no other compensation is owed to you except as provided herein.

6. **Non-Disparagement** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, for a period of five years following the date hereof you shall not make any false, disparaging or derogatory statements to any person or entity, including any media outlet, regarding the Company or any of its directors, officers, employees, agents or representatives or about the Company's business affairs and financial condition. Further, for a period of five years following the date hereof, neither the Company, nor any of its executive officers or members of its Board will directly or indirectly make, or cause to be made, any false statement, observation or opinion, disparaging your reputation.

7. **Continued Assistance** - You agree that after the Termination Date you will provide all reasonable cooperation to the Company, including but not limited to, assisting the Company transition your job duties, assisting the Company in defending against and/or prosecuting any litigation or threatened litigation, and performing any other tasks as reasonably requested by the Company.

8. **Cooperation** – To the extent permitted by law, you agree to cooperate fully with the Company in the defense or prosecution of any claims or actions which already have been brought, are currently pending, or which may be brought in the future against or on behalf of the Company**,** whether before a state or federal court, any state or federal government agency, or a mediator or arbitrator. Your full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare its claims or defenses, to prepare for trial or discovery or an administrative hearing or a mediation or arbitration and to act as a witness when requested by the Company at reasonable times designated by the Company. You agree that you will notify the Company promptly in the event that you are served with a subpoena or in the event that you are asked to provide a third party with information concerning any actual or potential complaint or claim against the Company.

9. **Amendment and Waiver** – This letter agreement shall be binding upon the parties and may not be modified in any manner, except by an instrument in writing of concurrent or subsequent date signed by duly authorized representatives of the parties hereto. This letter agreement is binding upon and shall inure to the benefit of the parties and their respective agents, assigns, heirs, executors, successors and administrators. No delay or omission by the Company in exercising any right under this letter agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar to or waiver of any right on any other occasion.

10. **Validity** – Should any provision of this letter agreement be declared or be determined by any court of competent jurisdiction to be illegal or invalid, the validity of the remaining parts, terms or provisions shall not be affected thereby and said illegal or invalid part, term or provision shall be deemed not to be a part of this letter agreement.

11. **Confidentiality** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, the terms and contents of this letter agreement, and the contents of the negotiations and discussions resulting in this letter agreement, shall be maintained as confidential by you and your agents and representatives and shall not be disclosed except to the extent required by federal or state law or as otherwise agreed to in writing by the Company.

12. **Nature of Agreement** – You understand and agree that this letter agreement is a severance agreement and does not constitute an admission of liability or wrongdoing on the part of the Company.

13. **Acknowledgments –** You acknowledge that you have been given at least [twenty-one (21) days] to consider this letter agreement, and that the Company advised you to consult with an attorney of your own choosing prior to signing this letter agreement. [You understand that you may revoke this letter agreement for a period of seven (7) days after you sign this letter agreement by notifying me in writing, and the letter agreement shall not be effective or enforceable until the expiration of this seven (7) day revocation period.] You understand and agree that by entering into this agreement, you are waiving any and all rights or claims you might have under the Age Discrimination in Employment Act, as amended by the Older Workers Benefits Protection Act, and that you have received consideration beyond that to which you were previously entitled.

14. **Voluntary Assent** – You affirm that no other promises or agreements of any kind have been made to or with you by any person or entity whatsoever to cause you to sign this letter agreement, and that you fully understand the meaning and intent of this letter agreement. You state and represent that you have had an opportunity to fully discuss and review the terms of this letter agreement with an attorney. You further state and represent that you have carefully read this letter agreement, understand the contents herein, freely and voluntarily assent to all of the terms and conditions hereof and sign your name of your own free act.

15. **Applicable Law** – This letter agreement shall be interpreted and construed by the laws of the State of New Jersey, without regard to conflict of laws provisions. You hereby irrevocably submit to and acknowledge and recognize the jurisdiction of the courts of the State of New Jersey, or if appropriate, a federal court located in the State of New Jersey (which courts, for purposes of this letter agreement, are the only courts of competent jurisdiction), over any suit, action or other proceeding arising out of, under or in connection with this letter agreement or the subject matter hereof.

16. **Entire Agreement** – This letter agreement contains and constitutes the entire understanding and agreement between the parties hereto with respect to your Severance Compensation and the settlement of claims against the Company and cancels all previous oral and written negotiations, agreements and commitments in connection therewith. Nothing in this paragraph, however, shall modify, cancel or supersede your obligations set forth in paragraph 3 herein.

17. **Tax Acknowledgement** – In connection with the payments and consideration provided to you pursuant to this letter agreement, the Company shall withhold and remit to the tax authorities the amounts required under applicable law, and you shall be responsible for all applicable taxes with respect to such payments and consideration under applicable law. You acknowledge that you are not relying upon the advice or representation of the Company with respect to the tax treatment of any of the Severance Compensation set forth in Section 4 of the Employment Agreement.

If you have any questions about the matters covered in this letter agreement, please call me at **[Insert Phone Number]**.

Very truly yours,

By: _____
[Name]
[Title]

I hereby agree to the terms and conditions set forth above. I have been given at least [twenty-one (21) days] to consider this letter agreement and I have chosen to execute this on the date below. I intend that this letter agreement will become a binding agreement between me and the Company [if I do not revoke my acceptance in seven (7) days].

_____ _____
[Insert Employee Name] Date

To be returned to me by **[Return Date – e.g., 21 days from date of receipt of this letter]**.

AMENDMENT TO EMPLOYMENT AGREEMENT

This Amendment (the "Amendment") is made by and between Marcio Silva de Souza ("Executive") and PTC Therapeutics, Inc. (the "Company") (collectively, "the Parties").

WHEREAS, Executive is employed by PTC pursuant to an Employment Agreement between Executive and PTC dated July 8, 2014 (the "Employment Agreement"); and

WHEREAS, on June 1, 2016 (the "Amendment Effective Date"), in connection with Executive's promotion, the Company is authorized to enter in to an amendment to Executive's Employment Agreement to reflect the title of Senior Vice President, with conforming changes to the Employment Agreement with respect to title, severance compensation, and annual bonus target;

NOW, THEREFORE, for good and valuable consideration, the sufficiency of which is acknowledged hereby, and in consideration of the mutual covenants and undertakings set forth herein, the Parties agree that the following sections of the Employment Agreement are amended and restated as follows as of the Amendment Effective Date:

Section 1(a), Capacity: "Executive shall serve the Company as Senior Vice President, Head of Product Strategy reporting to Mark Rothera or such other senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Senior Vice President, Head of Product Strategy. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his position as Senior Vice President, Head of Product Strategy as may be reasonably assigned to Executive from time to time by the individual to whom he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080, subject to relocation and Section 2(c)(i) of this Agreement."

Section 2(c), Definition of "Good Reason", clause (ii): "A material adverse change by the Company in Executive's duties, authority or responsibilities as Senior Vice President, Head of Product Strategy of the Company which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business."

Section 3(b), Bonus: In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 40% of Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be

paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned."

Section 4, Severance Compensation, subsection (b): "In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth below for twelve (12) months following Executive's termination of employment (the "Severance Period"). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution and nonrevocation of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing 30 days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his Base Salary for the Severance Period, which total amount shall be payable in a lump sum commencing no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) Only if Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his target annual bonus, described in section 3(b) hereof, for the year in which the termination of employment occurs, which total amount shall be payable in a lump sum commencing no later than sixty (60) days following Executive's termination of employment, provided that the Release has been executed and any applicable revocation period has expired as of such date.

(iii) The Company shall continue to provide Executive and his then-enrolled eligible dependents with group health insurance and shall continue to pay the amount of the premium as in effect on the date of such termination for the Severance Period commencing on the effective date of such termination, subject to applicable law and the terms of the respective policies; provided that the Company's obligation to provide the benefits contemplated herein shall terminate upon Executive's becoming eligible for coverage under the medical benefits program of a subsequent employer. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law. ";

With the following subsections of subsection (4)(b) to be renumbered and referenced consistent with the insertion of the new subsection (4)(b)(ii) set forth above and the renumbering of the prior subsection 4(b)(ii) as subsection 4(b)(iii).

No Other Changes. Except as explicitly provided above, the Parties agree that there are no other changes or amendments to the Employment Agreement and that the Employment Agreement, as amended by this Amendment, remains in full force and effect.

AGREED AND ACCEPTED

PTC Therapeutics, Inc.

By: _/s/ Mark Boulding_____

Name: Mark Boulding

Title: EVP, Chief Legal Officer

Date: __September 23, 2016_____

AGREED AND ACCEPTED:

Marcio Silva de Souza

By: _/s/ Marcio Silva de Souza ____

Date: __June 10, 2016_____

May 31, 2017

Marcio Souza
7 Lenore
Califon, NJ 07830

Dear Marcio,

Congratulations on your appointment to Chief Operating Officer. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's success. The effective date of your appointment is May 31, 2017. In this new role you will report to CEO and Founder, Stu Peltz.

Outlined below are details of your appointment, subject to your acceptance:

- Your annual base salary will be increased to $435,000 annually, subject to deductions for taxes and other withholdings as required by law. This represents an increase of 18.8% over your current base salary.

- Your bonus target will continue to be 40.00% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's annual incentive compensation plan.

- You are eligible to receive a one-time appointment bonus of $75,000, payable with acceptance of this letter. Payment will be made as soon as practical taking into account the announcement of the appointment and payroll cycle.

- You are eligible to receive a retention bonus equal to your bonus target at 100% payout (approximately $174,000), payable in a lump sum, if you remain employed with PTC for a period of one year from the effective date of this appointment.

- You will receive a one-time grant of 30,000 stock options to purchase shares of common stock of PTC, and a one-time grant of 30,000 shares of Restricted Stock Awards, vesting equally in two tranches one year and two years after the grant date, subject to formal approval by the Compensation Committee of the Board of Directors. Note that this is equivalent to 105,000 option equivalents.

On behalf of PTC, let me again congratulate you on your appointment. We look forward to this next step in your career. Please feel free to contact me if you have any questions concerning your appointment.

Sincerely,

/s/ Martin Rexroad
Martin Rexroad
SVP, Human Resources

Accepted by:

/s/ Marcio Souza
Marcio Souza
May 31, 2017

Cc: Stu Peltz

EMPLOYMENT AGREEMENT

This EMPLOYMENT AGREEMENT (the "Agreement") is made as of August 16, 2014 (the "Effective Date"), by and between PTC Therapeutics, Inc., a Delaware corporation (the "Company") and Christine Utter ("Executive"). In consideration of the mutual covenants contained in this Agreement, the Company and Executive agree as follows:

1. Employment. The Company agrees to continue to employ Executive and Executive agrees to continue to be employed by the Company on the terms and conditions set forth in this Agreement.

(a) Capacity. Executive shall serve the Company as Vice President, Finance reporting to Shane Kovacs, CFO, or such other senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Vice President, Finance. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his/her position as Vice President, Finance as may be reasonably assigned to Executive from time to time by the individual to whom s/he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080, subject to relocation and Section 2(c)(i) of this Agreement.

(b) Devotion of Duties; Representations. During the Term (as defined below) of Executive's employment with the Company, Executive shall devote his/her best efforts and full business time and energies to the business and affairs of the Company, and shall endeavor to perform the duties and services contemplated hereunder to the reasonable satisfaction of the individual to whom s/he reports and the Board. During the Term of Executive's employment with the Company, Executive shall not, without the prior written approval of the Company (by action of the Board), undertake any other employment from any person or entity or serve as a director of any other company; provided, however, that (i) the Company will entertain requests as to such other employment or directorships in good faith and (ii) Executive will be eligible to participate in any policy relating to outside activities that is applicable to the senior executives of the Company and approved by the Board after the date hereof.

2. Term of Employment.

(a) Executive's employment hereunder shall continue on the Effective Date. Executive's employment hereunder shall be terminated upon the first to occur of the following:

(i) Immediately upon Executive's death;

(ii) By the Company:

(A) By written notice to Executive effective the date of such notice, following the Disability of Executive. "Disability" means that Executive (i) is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or can be expected to last for a continuous period of not less than

12 months, or (ii) is, by reason of any medically determinable physical or mental impairment which can be expected to last for a continuous period of not less than 12 months, receiving income replacement benefits for a period of not less than three months under an accident and health plan covering employees of the Company. Such incapacity shall be determined by a physician chosen by the Company and reasonably satisfactory to Executive (or Executive's legal representative) upon examination requested by the Company (to which Executive hereby agrees to submit). Notwithstanding the foregoing, such Disability must result in Executive becoming "Disabled" within the meaning of Section 409A(a)(2)(C) of the Internal Revenue Code of 1986, as amended (the "Code") and the guidance issued thereunder. (In this Agreement we refer to Section 409A of the Code and any guidance issued thereunder as "Section 409A").

(B) By written notice to Executive, effective the date of such notice, for Cause (as defined below); or

(C) By written notice to Executive, effective ninety (90) days after the date of such notice and subject to Section 4 hereof, without Cause; or

(iii) By Executive:

(A) At any time by written notice to the Company, effective forty-five (45) days after the date of such notice**;** or

(B) By written notice to the Company for Good Reason (as defined below), effective on the date specified in such notice.

The term of Executive's employment by the Company under this Agreement is referred to herein as the "Term."

(b) Definition of "Cause". For purposes of this Agreement, "Cause" shall, pursuant to the reasonable good faith determination by a majority of the Board (excluding Executive) as documented in writing, include: (i) the willful and continued failure by Executive to substantially perform Executive's material duties or responsibilities under this Agreement (other than such a failure as a result of Disability); (ii) any action or omission by Executive involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) Executive's conviction of a felony, either in connection with the performance of Executive's obligations to the Company or which otherwise shall adversely affect Executive's ability to perform such obligations or shall materially adversely affect the business activities, reputation, goodwill or image of the Company; (iv) the material breach of a fiduciary duty to the Company; or (v) the material breach by Executive of any of the provisions of this Agreement, provided that any breach of Executive's obligations with respect to Sections 5 or 6 of this Agreement, subject to the cure provision in the next sentence, shall be deemed "material." In respect of the events described in clauses (i) and (v) above, the Company shall give Executive notice of the failure of performance or breach, reasonable as to time, place and manner in the circumstances, and a 30-day opportunity to cure, provided that such failure of performance or breach is reasonably amenable to cure as determined by the Board in its sole discretion.

(c) Definition of "Good Reason". For purposes of this Agreement, a "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from Executive within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Executive has consented to the condition that would otherwise be a basis for Good Reason:

(i) A change in the principal location at which Executive provides services to the Company to a location more than fifty (50) miles from such principal location and/or to a location in New York City (either of which change, the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which Executive provides services to the Company), provided that such a relocation shall not be deemed to occur under circumstances where Executive's responsibilities require him/her to work at a location other than the corporate headquarters for a reasonable period of time;

(ii) A material adverse change by the Company in Executive's duties, authority or responsibilities as Vice President, Finance of the Company which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business.

(iii) A material reduction in Executive's base compensation (including Base Salary) except if the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change;

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by Executive; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

(d) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the Securities Exchange Act of 1934 (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or

substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) Executive forms part of an executive management team that consists of substantially the same group of individuals and Executive is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

3. Compensation.

(a) Base Salary. Executive's minimum base salary during the Term shall be at the rate of $220,000 per year (the "Base Salary"). Base Salary shall be payable in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, less any amounts required to be withheld under applicable law. The Base Salary will be subject to adjustment from time to time in the sole discretion of the Board; provided that, the Company covenants that it shall not reduce the Base Salary below $220,000 or the Base Salary then in effect immediately prior to the reduction unless (i) Executive consents to such reduction, or (ii) the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change.

(b) Bonus. In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 30% of Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned.

(c) Equity Compensation. Except as explicitly set forth below, Executive's rights with respect to equity (including stock options) shall be covered in PTC's equity and long term incentive plan(s) and separate stock option certificates or agreements for each grant.

(i) Accelerated Vesting.

(A) For the avoidance of doubt, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason, no unvested equity awards granted under the Company's equity and long-term incentive plan(s) following the date hereof shall be subject to any accelerated vesting except as otherwise provided for in the applicable award agreement or in Section 3(c)(i)(B) below.

(B) Except as otherwise provided in the applicable award, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs during the Term (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of all of Executive's outstanding unvested equity awards granted under the Company's equity and long-term incentive plan(s) following the date hereof shall vest immediately.

(d) Vacation. Executive is eligible for time off programs outlined in the Company's Time Off Policy. Executive shall accrue over the calendar year 160 hours of paid vacation. Executive may accrue up to 200 hours of vacation. Once Executive has reached the maximum accrual, no further vacation time will be accrued unless and until the Executive uses vacation time. Upon termination of employment, the value of Executive's current balance of accrued but unused vacation shall be paid out in cash based on his/her Base Salary that was in effect immediately prior to his/her termination of employment.

(e) Fringe Benefits. Executive shall be entitled to participate in any employee benefit plans that the Company makes available to its senior executives (including, without limitation, group life, disability, medical, dental and other insurance, retirement, pension, profit-sharing and similar plans) (collectively, the "Fringe Benefits"), provided that the Fringe Benefits shall not include any stock option or similar plans relating to the grant of equity securities of the Company. These benefits may be modified or changed from time to time at the sole discretion of the Company. Where a particular benefit is subject to a formal plan (for example, medical or life insurance), eligibility to participate in and receive any particular benefit is governed solely by the applicable plan document, and eligibility to participate in such plan(s) may be dependent upon, among other things, a physical examination.

(f) Reimbursement of Expenses. Executive shall be entitled to reimbursement for all ordinary and reasonable out-of-pocket business expenses that are reasonably incurred by him/her in furtherance of the Company's business in accordance with reasonable policies adopted from time to time by the Company for senior executives.

(g) Taxes and Withholdings. The Company shall deduct and withhold from all compensation and benefits under this Agreement all social security and other federal, state and local taxes and charges which currently are or which hereafter may be required by law to be so deducted and withheld.

4. Severance Compensation.

(a) In the event of any termination of Executive's employment for any reason the Company shall pay Executive (or Executive's estate) such portions of Executive's Base Salary as have accrued prior to such termination and have not yet been paid, together with (i) amounts for accrued unused vacation days (as provided above), (ii) any amounts for expense reimbursement which have been properly incurred or the Company has become obligated to pay prior to termination and have not been paid as of the date of such termination and (iii) the amount of any Bonus previously granted to Executive by the Board but not yet paid, which amount shall not include any pro rata portion of any Bonus which would have been earned if such termination had not occurred (the "Accrued Obligations"). Such amounts shall be paid as soon as possible after termination.

(b) In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth below for six (6) months following Executive's termination of employment (the "Severance Period"). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution (and, as applicable, non-revocation) of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his/her Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing no later than sixty (60) days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his/her Base Salary for the Severance Period, which total amount shall be payable in a lump sum no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) The Company shall continue to provide Executive and his/her then-enrolled eligible dependents with group health insurance and shall continue to pay the amount of the premium as in effect on the date of such termination for the Severance Period commencing on the effective date of such termination, subject to applicable law and the terms of the respective policies; provided that the Company's obligation to provide the benefits contemplated herein shall terminate upon Executive's becoming eligible for coverage under the medical benefits program of a subsequent employer. The

foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.

(iii) Compliance with Section 409A. Subject to the provisions in this Section 4(b)(iii), any severance payments or benefits under this Agreement shall begin only upon the date of Executive's "separation from service" (determined as set forth below) which occurs on or after the date of termination of Executive's employment. The following rules shall apply with respect to the distribution of the severance payments and benefits, if any, to be provided to Executive under this Agreement:

(1) It is intended that each installment of the severance payments and benefits provided under this Agreement shall be treated as a separate "payment" for purposes of Section 409A. Neither the Company nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(2) If, as of the date of Executive's "separation from service" from the Company, Executive is not a "specified employee" (within the meaning of Section 409A), then each installment of the severance payments and benefits shall be made on the dates and terms set forth in this Agreement.

(3) If, as of the date of Executive's "separation from service" from the Company, Executive is a "specified employee" (within the meaning of Section 409A), then:

(A) Each installment of the severance payments and benefits due under this Agreement that, in accordance with the dates and terms set forth herein, will in all circumstances, regardless of when the separation from service occurs, be paid within the short-term deferral period (as defined under Section 409A) shall be treated as a short-term deferral within the meaning of Treasury Regulation Section 1.409A-1(b)(4) to the maximum extent permissible under Section 409A and such payments and benefits shall be paid or provided on the dates and terms set forth in this Agreement; and

(B) Each installment of the severance payments and benefits due this Agreement that is not described in Section 4(b)(iii)(3)(A) above and that would, absent this subsection, be paid within the six-month period following Executive's "separation from service" from the Company shall not be paid until the date that is six months and one day after such separation from service (or, if earlier, Executive's death), with any such installments that are required to be delayed being accumulated during the six-month period and paid in a lump sum on the date that is six months and one day following Executive's separation from service and any subsequent installments, if any, being paid in accordance with the dates and terms set forth herein; provided, however, that the preceding provisions of this sentence shall not apply to any installment of severance payments and benefits if and to the maximum extent that such installment is deemed to be paid under a separation pay plan that does not provide

for a deferral of compensation by reason of the application of Treasury Regulation 1.409A-1(b)(9)(iii) (relating to separation pay upon an involuntary separation from service). Any installments that qualify for the exception under Treasury Regulation Section 1.409A-1(b)(9)(iii) must be paid no later than the last day of Executive's second taxable year following the taxable year in which the separation from service occurs.

(4) The determination of whether and when Executive's separation from service from the Company has occurred shall be made in a manner consistent with, and based on the presumptions set forth in, Treasury Regulation Section 1.409A-1(h). Solely for purposes of this Section 4(b)(iii), "Company" shall include all persons with whom the Company would be considered a single employer under Section 414(b) and 414(c) of the Code.

(5) All reimbursements and in-kind benefits provided under this Agreement shall be made or provided in accordance with the requirements of Sections 409A to the extent that such reimbursements or in-kind benefits are subject to Section 409A, including, where applicable, the requirements that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement), (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses eligible for reimbursement in any other calendar year, (iii) the reimbursement of an eligible expense will be made on or before the last day of the calendar year following the year in which the expense is incurred and (iv) the right to reimbursement is not subject to set off or liquidation or exchange for any other benefit.

(6) Notwithstanding anything herein to the contrary, the Company shall have no liability to Executive or to any other person if the payments and benefits provided hereunder that are intended to be exempt from or compliant with Section 409A are not so exempt or compliant.

(c) In the event that Executive's employment hereunder is terminated (i) by Executive for other than a Good Reason, or (ii) by the Company for Cause, or (iii) as a result of Executive's death or Disability, then the Company will pay to Executive the Accrued Obligations. The Company shall have no obligation to pay Executive (or Executive's estate) any other compensation following such termination except as provided in Section 4(a).

(d) Modified Section 280G Cutback.

(i) Notwithstanding any other provision of this Agreement, except as set forth in Section 4(d)(ii), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide to Executive a portion of any "Contingent Compensation Payments" (as defined below) that Executive would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for Executive. For purposes of this Section 4(d), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount

(determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(ii) Notwithstanding the provisions of Section 4(d)(i), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by Executive if the Eliminated Payments (determined without regard to this sentence) were paid to him/her (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of Executive's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 4(d)(ii) shall be referred to as a "Section 4(d)(ii) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(iii) For purposes of this Section 4(d) the following terms shall have the following respective meanings:

(1) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(2) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(iv) Any payments or other benefits otherwise due to Executive following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 4(d)(iv). Within 30 days after each date on which Executive first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify Executive (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 4(d)(ii) Override is applicable. Within 30 days after delivery of such notice to Executive, Executive shall deliver a response to the Company (the "Executive

Response") stating either (A) that s/he agrees with the Company's determination pursuant to the preceding sentence or (B) that s/he disagrees with such determination, in which case s/he shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 4(d)(ii) Override is applicable. In the event that Executive fails to deliver an Executive Response on or before the required date, the Company's initial determination shall be final. If Executive states in the Executive Response that s/he agrees with the Company's determination, the Company shall make the Potential Payments to Executive within three business days following delivery to the Company of the Executive Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If Executive states in the Executive Response that s/he disagrees with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, Executive and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in South Plainfield, New Jersey, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to Executive those Potential Payments as to which there is no dispute between the Company and Executive regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(v) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by Executive for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by Executive in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and

not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(vi) The provisions of this Section 4(d) are intended to apply to any and all payments or benefits available to Executive under this Agreement or any other agreement or plan of the Company under which Executive receives Contingent Compensation Payments.

(vii) Notwithstanding Sections 4(d)(i)-(vi) hereof, until the closing of the first underwritten public offering of common stock of the Company, in the event that it shall be determined that any payment or benefit (including any accelerated vesting of options or other equity awards) made or provided, or to be made or provided, by the Company (or any successor thereto or affiliate thereof) to or for the benefit of Executive, whether pursuant to the terms of this Agreement, any other agreement, plan, program or arrangement of or with the Company (or any successor thereto or affiliate thereof) or otherwise, may be subject to the excise tax imposed by Section 4999 of the Code or any comparable tax imposed by any replacement or successor provision of United States tax law, then upon the request of Executive, the Company shall use reasonable efforts to procure a shareholder vote in satisfaction of the shareholder approval requirements described in Treas. Reg. Section 1.280G-1, Q&A-7.

5. Executive Covenants.

(a) Confidential Information. Executive recognizes and acknowledges the competitive and proprietary aspects of the business of the Company, and that as a result of Executive's employment, Executive recognizes and acknowledges that s/he has had and will continue to have access to, and has been and will continue to be involved in the development of, Confidential Information (as defined below) of the Company. As used herein, "Confidential Information" shall mean and include trade secrets, knowledge and other confidential information of the Company, which Executive has acquired, no matter from whom or on what matter such knowledge or information may have been acquired, heretofore or hereafter, concerning the content and details of the business of the Company, and which is not known to the general public, including but not limited to: (a) new products, product betterments and other inventions, formulas, processes, methods, materials, material combinations, manner of preparations, technical production procedures and information, alarm and security codes and procedures, sources of technology, and sources of supply of raw and finished materials and other products; (b) financial and accounting records; (c) the identity of employees, consultants, independent contractors, customers, business development partners, licensees, suppliers, creditors or other parties with which the Company has business dealings, the nature of the relationship with such persons, or any other information relating to such persons or the Company's dealings with such persons; and (d) computer software used by the Company or provided to the customers of the Company unless publicly available.

(i) For as long as Executive is employed and at all times thereafter, Executive shall not, directly or indirectly, communicate, disclose or divulge to any person or entity, or use for Executive's own benefit or the benefit of any person (other than the Company), any Confidential Information, except as permitted in subparagraph (iii)

below. Upon termination of Executive's employment, or at any other time at the request of the Company, Executive agrees to deliver promptly to the Company all Confidential Information, including, but not limited to, customer and supplier lists, files and records, in Executive's possession or under Executive's control. Executive further agrees that s/he will not make or retain any copies of any of the foregoing and will so represent to the Company upon termination of Executive's employment.

(ii) Executive shall disclose immediately to the Company any trade secrets or other Confidential Information conceived or developed by Executive at any time during Executive's employment. Executive hereby assigns and agrees to assign to the Company Executive's entire right, title and interest in and to all Confidential Information. Such assignment shall include, without limitation, the rights to obtain patent or copyright protection, thereon in the United States and foreign countries. Executive agrees to provide all reasonable assistance to enable the Company to prepare and prosecute any application before any governmental agency for patent or copyright protection or any similar application with respect to any Confidential Information. Executive further agrees to execute all documents and assignments and to make all oaths necessary to vest ownership of such intellectual property rights in the Company, as the Company may request. These obligations shall apply whether or not the subject thereof was conceived or developed at the suggestion of the Company, and whether or not developed during regular hours of work or while on the premises of the Company.

(iii) Executive shall at all times, both during and after termination of this Agreement by either Executive or the Company, maintain in confidence and shall not, without prior written consent of the Company, use, except in the course of performance of Executive's duties for the Company or as required by legal process (provided that Executive will promptly notify the Company of such legal process except with respect to any confidential government investigation), disclose or give to others any Confidential Information. In the event Executive is questioned by anyone not employed by the Company or by an employee of or a consultant to the Company not authorized to receive such information, in regard to any such information or any other secret or confidential work of the Company, or concerning any fact or circumstance relating thereto, Executive will promptly notify the Company.

(b) Non-Competition and Non-Solicitation. Executive recognizes that the Company is engaged in a competitive business and that the Company has a legitimate interest in protecting its trade secrets, confidential business information, and customer, business development partner, licensee, supplier, and credit and/or financial relationships. Accordingly, in exchange for valuable consideration, including without limitation Executive's access to confidential business information and continued at-will employment, Executive agrees that, during the Term hereof and for a period of eighteen (18) months thereafter, Executive shall not:

(i) directly or indirectly, whether for himself or for any other person or entity, and whether as a proprietor, principal, shareholder, partner, agent, employee, consultant, independent contractor, or in any other capacity whatsoever, undertake or have any interest in (other than the passive ownership of publicly registered securities representing an ownership interest of less than 1%), engage in or assume any role

involving directly or indirectly the Company's Field of Interest (or any portion thereof) or any other business in which the Company is engaged and for which the employee has rendered services while employed by the Company, or enter into any agreement to do any of the foregoing; or

(ii) initiate contact with (including without limitation phone calls, press releases and the sending or delivering of announcements), or in any manner solicit, directly or indirectly, any customers, business development partners, licensors, licensees, or creditors (including institutional lenders, bonding companies and trade creditors) of the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to transfer any of their business with the Company to any person or entity other than the Company; or

(iii) initiate contact with, or in any manner solicit, directly or indirectly, any supplier of goods, services or materials to the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to supply the same or similar inventory, goods, services or materials (except generally available inventory, goods, services or materials) to any person or entity other than the Company; or

(iv) directly or indirectly recruit, solicit or otherwise induce or influence any employee or independent contractor of the Company to discontinue or modify his or her employment or engagement with the Company, or employ or contract with any such employee or contractor for the provision of services.

(c) Definition of "Field of Interest". The term Company's "Field of Interest" shall mean the research, development and commercialization of products and strategies relating to: (i) therapies for genetic disorders or diseases that include cystic fibrosis, Duchenne muscular dystrophy, other diseases caused in whole or part by nonsense (or stop) codons, and other genetic diseases as to which the Company engages in the research, development or commercialization of drugs; anti-angiogenic therapies that target VEGF protein production for cancer; and antiviral therapies for the Hepatitis C virus (HCV); and (ii) other therapeutic targets, mechanisms of action and/or therapies in which the Company has a research, development or commercialization program.

(d) Definition of "Customer". The term "customer" or "customers" shall include any person or entity (a) that is a current customer of the Company, (b) that was a customer of the Company at any time during the preceding twenty-four (24) months or (c) to which the Company made a written presentation for the solicitation of business at any time during the preceding twenty-four (24) months.

(e) Reasonableness of Restrictions. Executive further recognizes and acknowledges that (i) the types of employment which are prohibited by this Section 5 are narrow and reasonable in relation to the skills which represent Executive's principal salable asset both to the Company and to Executive's other prospective employers, and (ii) the broad geographical scope of the provisions of this Section 5 is reasonable, legitimate and fair to Executive in light of the global nature of the Company's business, particularly pharmaceutical research and development, and in light of the limited restrictions on the type of employment prohibited herein

compared to the types of employment for which Executive is qualified to earn Executive's livelihood.

(f) Remedies. Executive acknowledges that a breach of this Section 5 will cause great and irreparable injury and damage, which cannot be reasonably or adequately compensated by money damages. Accordingly, Executive acknowledges that the remedies of injunction and specific performance shall be available in the event of such a breach, in addition to money damages, costs and attorneys' fees, and other legal or equitable remedies, and that the Company shall be entitled as a matter of course to an injunction pending trial, without the posting of bond or other security. Any period of restriction set forth in this Section 5 shall be extended for a period of time equal to the duration of any breach or violation hereof.

(g) Notification. Any person employing Executive or evidencing any intention to employ Executive may be notified as to the existence and provisions of this Agreement.

(h) Modification of Covenants; Enforceability. In the event that any provision of this Section 5 is held to be in any respect an unreasonable restriction, then the court so holding may modify the terms thereof, including the period of time during which it operates or the geographic area to which it applies, or effect any other change to the extent necessary to render this section enforceable, it being acknowledged by the parties that the representations and covenants set forth herein are of the essence of this Agreement.

(i) Subsidiaries. For purposes of Sections 5 and 6 of this Agreement, "Company" shall include all direct and indirect subsidiaries of the Company. An entity shall be deemed to be a subsidiary of the Company if the Company directly or indirectly owns or controls 50% or more of the equity interest in such entity.

6. Ownership of Ideas, Copyrights and Patents.

(a) Property of the Company. Executive agrees that all ideas, discoveries, creations, manuscripts and properties, innovations, improvements, know-how, inventions, designs, developments, apparatus, techniques, methods, biological processes, cell lines, laboratory notebooks and formulae, whether patentable, copyrightable or not, which Executive may conceive, reduce to practice or develop, alone or in conjunction with another, or others, whether during or out of regular business hours, and whether at the request or upon the suggestion of the Company, or otherwise, in the course of performing services for the Company in any capacity, whether heretofore or hereafter, (collectively, "the Inventions") are and shall be the sole and exclusive property of the Company, and that Executive shall not publish any of the Inventions without the prior written consent of the Company. Executive hereby assigns to the Company all of Executive's right, title and interest in and to all of the foregoing. Executive further represents and agrees that to the best of Executive's knowledge and belief none of the Inventions will violate or infringe upon any right, patent, copyright, trademark or right of privacy, or constitute libel or slander against or violate any other rights of any person, firm or corporation and that Executive will use his/her best efforts to prevent any such violation.

(b) Cooperation. At any time during or after the Term, Executive agrees that s/he will fully cooperate with the Company, its attorneys and agents in the preparation and filing

of all papers and other documents as may be required to perfect the Company's rights in and to any of such Inventions, including, but not limited to, executing any lawful document (including, but not limited to, applications, assignments, oaths, declarations and affidavits) and joining in any proceeding to obtain letters patent, copyrights, trademarks or other legal rights of the United States and of any and all other countries on such Inventions, provided that any patent or other legal right so issued to Executive, personally, shall be assigned by Executive to the Company without charge by Executive. Executive further designates the Company as his/her agent for, and grants to the Company a power of attorney with full power of substitution, which power of attorney shall be deemed coupled with an interest, for the purpose of effecting the foregoing assignments from Executive to the Company. Company will bear the reasonable expenses which it causes to be incurred in Executive's assisting and cooperating hereunder. Executive waives all claims to moral rights in any Inventions.

7. Disclosure to Future Employers. The Company may provide in its discretion, a copy of the covenants contained in Sections 5 and 6 of this Agreement to any business or enterprise which Executive may directly, or indirectly, own, manage, operate, finance, join, control or in which Executive participates in the ownership, management, operation, financing, or control, or with which Executive may be connected as an officer, director, employee, partner, principal, agent, representative, consultant or otherwise.

8. Records. Upon termination of Executive's relationship with the Company, Executive shall deliver to the Company any property of the Company which may be in Executive's possession including products, materials, memoranda, notes, records, reports, or other documents or photocopies of the same.

9. Insurance. The Company, in its sole discretion, may apply for and procure in its own name (whether or not for its own benefit) policies of insurance insuring Executive's life. Executive agrees to submit to reasonable medical or other examinations and to execute and deliver any applications or other instruments in writing that are reasonably necessary to effectuate such insurance. No adverse employment actions may be based upon the results of any such exam or the failure by the Company to obtain such insurance.

10. No Conflicting Agreements. Executive hereby represents and warrants that Executive has no commitments or obligations inconsistent with this Agreement.

11. "Market Stand-Off" Agreement. Executive agrees, if requested by the Company and an underwriter of common stock (or other securities) of the Company, not to sell or otherwise transfer or dispose of any common stock (or other securities) of the Company held by Executive during a period not to exceed one hundred and eighty (180) days following the effective date of the first underwritten public offering of common stock of the Company, offered on a firm commitment basis pursuant to a registration statement filed with the Securities and Exchange Commission (or any successor agency of the Federal government administrating the Securities Act of 1933, as amend, and the Securities Exchange Act of 1934, as amended) under the Securities Act of 1933, as amended, on Form S-1 or its then equivalent, and to enter into an agreement to such effect. The Company may impose stop-transfer instructions with respect to the shares (or securities) subject to the foregoing restriction until the end of said period.

12. General.

(a) Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address as follows:

If to the Company: PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080
USA
Attention: Legal Department
Telephone: (908) 222-7000

With an email copy to: legal@ptcbio.com

If to Executive: Christine Utter
3 Pembroke Court
Marlboro, NJ 07746

or to such other address as a party may designate by notice hereunder, and shall be either (i) delivered by hand, (ii) sent by overnight courier, or (iii) sent by registered or certified mail, return receipt requested, postage prepaid. All notices, requests, consents and other communications hereunder shall be deemed to have been given either (i) if by hand, at the time of the delivery thereof to the receiving party at the address of such party set forth above, (ii) if sent by overnight courier, on the next business day following the day such notice is delivered to the courier service, or (iii) if sent by registered or certified mail, on the fifth (5th) business day following the day such mailing is made.

(b) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, except with respect to the equity and fringe benefit arrangements referred to in Subsections 3(c) and (e) above. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(c) Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(d) Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(e) Assignment. The Company shall assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of the Company's business or that aspect of the Company's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of the Company.

(f) Benefit. All statements, representations, warranties, covenants and agreements in this Agreement shall be binding on the parties hereto and shall inure to the benefit of the respective successors and permitted assigns of each party hereto. Nothing in this Agreement shall be construed to create any rights or obligations except among the parties hereto, and no person or entity shall be regarded as a third-party beneficiary of this Agreement.

(g) Governing Law. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the law of The State of New Jersey, without giving effect to the conflict of law principles thereof.

(h) Jurisdiction and Service of Process. Any legal action or proceeding with respect to this Agreement shall be brought in the courts of The State of New Jersey or of the United States of America for the District of New Jersey. By execution and delivery of this Agreement, each of the parties hereto accepts for itself and in respect of its property, generally and unconditionally, the jurisdiction of the aforesaid courts. Each of the parties hereto irrevocably consents to the service of process of any of the aforementioned courts in any such action or proceeding by the mailing of copies thereof by certified mail, postage prepaid, to the party at its address set forth in Section 12(a) hereof.

(i) Severability. The parties intend this Agreement to be enforced as written. However, (i) if any portion or provision of this Agreement shall to any extent be declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby or otherwise, the Company and Executive agrees that the court making such determination shall have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases ("blue-penciling"), and in its reduced or blue-penciled form such provision shall then be enforceable and shall be enforced.

(j) Headings and Captions; Interpretation. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify, or affect the meaning or construction of any of the terms or provisions hereof. The provisions of the following Sections of this Agreement are in addition to, and do not limit, each other: Sections 6 and 5(a); Sections 7 and 5(g); Sections 12(k) and 12(f); and Sections 12(l) and 12(d).

(k) Injunctive Relief. Executive hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Section 5 or 6 of this Agreement will result in substantial, continuing and irreparable injury to the Company.

Therefore, Executive hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction.

(l) No Waiver of Rights, Powers and Remedies. No failure or delay by a party hereto in exercising any right, power or remedy under this Agreement, and no course of dealing between the parties hereto, shall operate as a waiver of any such right, power or remedy of the party. No single or partial exercise of any right, power or remedy under this Agreement by a party hereto, nor any abandonment or discontinuance of steps to enforce any such right, power or remedy, shall preclude such party from any other or further exercise thereof or the exercise of any other right, power or remedy hereunder. The election of any remedy by a party hereto shall not constitute a waiver of the right of such party to pursue other available remedies. No notice to or demand on a party not expressly required under this Agreement shall entitle the party receiving such notice or demand to any other or further notice or demand in similar or other circumstances or constitute a waiver of the rights of the party giving such notice or demand to any other or further action in any circumstances without such notice or demand.

(m) Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

(n) Survival. The provisions of Sections 4, 5, 6, 7, 8, 11 and 12 shall survive the termination of this Agreement and Executive's employment hereunder in accordance with their terms.

(o) WAIVER OF TRIAL BY JURY. THE PARTIES IRREVOCABLY WAIVE THEIR RESPECTIVE RIGHT TO A TRIAL BY JURY REGARDING ANY DISPUTE, CLAIM OR CAUSE OF ACTION ARISING OUT OF, CONCERNING, OR RELATED TO EXECUTIVE'S EMPLOYMENT WITH THE COMPANY OR THIS AGREEMENT.

(p) Knowing and Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement knowingly and voluntarily and without any duress or undue influence by PTC or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and fully understands it, including that Executive is waiving the right to a jury trial. Executive further agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

IN WITNESS THEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

/s/ Mark Boulding
Name: Mark Boulding
Title: Executive Vice President & Chief Legal Officer

Agreed and Accepted

/s/ Christine Utter
Name: Christine Utter

EXHIBIT A

Sample Separation and Release Agreement

[Insert Date]

[Insert Employee Name]
[Insert Employee Address]

Dear **[Insert Employee Name]**:

In connection with the termination of your employment with PTC Therapeutics, Inc. (the "Company") on **[Termination Date]**, you are eligible to receive the Severance Compensation as described in Section 4 of the Employment Agreement executed between you and the Company on [**Insert Date**] (the "Employment Agreement") if you sign and return this letter agreement to me by **[Return Date – e.g., 21 days from date of receipt of this letter agreement]** and it becomes binding between you and the Company. By signing and returning this letter agreement [and not revoking your acceptance], you will be agreeing to the terms and conditions set forth in the numbered paragraphs below, including the release of claims set forth in paragraph 3. Therefore, you are advised to consult with an attorney before signing this letter agreement and you may take up to [twenty-one (21) days] to do so. [If you sign this letter agreement, you may change your mind and revoke your agreement during the seven (7) day period after you have signed it by notifying me in writing. If you do not so revoke, this letter agreement will become a binding agreement between you and the Company upon the expiration of the seven (7) day period.]

If you choose not to sign and return this letter agreement by **[Return Date-Same as Above][**, or if you timely revoke your acceptance in writing], you shall not receive any Severance Compensation from the Company. You will, however, receive payment for your final wages and any unused vacation time accrued through the Termination Date, as defined below, on the Company's regular payroll date immediately following the Termination Date. Also, regardless of signing this letter agreement, you may elect to continue receiving group medical insurance pursuant to the federal "COBRA" law, 29 U.S.C. § 1161 et seq. If you so elect, you shall pay all premium costs on a monthly basis for as long as, and to the extent that, you remain eligible for COBRA continuation. You should consult the COBRA materials to be provided by the Company for details regarding these benefits. All other benefits will cease upon your Termination Date in accordance with the plan documents.

The following numbered paragraphs set forth the terms and conditions that will apply if you timely sign and return this letter agreement and do not revoke it in writing within the seven (7) day period.

1. **Termination Date** – Your effective date of termination from the Company is **[Insert Date]** (the "Termination Date").

2. **Release** – In consideration of the payment of the Severance Compensation, which you acknowledge you would not otherwise be entitled to receive, you hereby fully, forever,

irrevocably and unconditionally release, remise and discharge the Company, its affiliates, subsidiaries, parent companies, predecessors, and successors, and all of their respective past and present officers, directors, stockholders, partners, members, employees, agents, representatives, plan administrators, attorneys, insurers and fiduciaries (each in their individual and corporate capacities) (collectively, the "Released Parties") from any and all claims, charges, complaints, demands, actions, causes of action, suits, rights, debts, sums of money, costs, accounts, reckonings, covenants, contracts, agreements, promises, doings, omissions, damages, executions, obligations, liabilities, and expenses (including attorneys' fees and costs), of every kind and nature that you ever had or now have against any or all of the Released Parties, including, but not limited to, any and all claims arising out of or relating to your employment with and/or separation from the Company, including, but not limited to, all claims under Title VII of the Civil Rights Act of 1964, 42 U.S.C. § 2000e et seq., the Americans With Disabilities Act of 1990, 42 U.S.C. § 12101 et seq., the Age Discrimination in Employment Act, 29 U.S.C. § 621 et seq., the Genetic Information Nondiscrimination Act of 2008, 42 U.S.C. § 2000ff et seq., the Family and Medical Leave Act, 29 U.S.C. § 2601 et seq., the Worker Adjustment and Retraining Notification Act ("WARN"), 29 U.S.C. § 2101 et seq., the Rehabilitation Act of 1973, 29 U.S.C. § 701 et seq., Executive Order 11246, Executive Order 11141, the Fair Credit Reporting Act, 15 U.S.C. § 1681 et seq., and the Employee Retirement Income Security Act of 1974 ("ERISA"), 29 U.S.C. § 1001 et seq., all as amended; all claims arising out of the New Jersey Law Against Discrimination, N.J. Stat. Ann. § 10:5-1 et seq., the New Jersey Family Leave Act, N.J. Stat. Ann. § 34:11B-1 et seq., the New Jersey Conscientious Employee Protection Act, N.J. Stat. Ann. § 34:19-1 et seq., and the N.J. Stat. Ann. § 34:11-56.1 et seq. (New Jersey equal pay law), all as amended; all common law claims including, but not limited to, actions in defamation, intentional infliction of emotional distress, misrepresentation, fraud, wrongful discharge, and breach of contract, including without limitation, all claims arising from the Employment Agreement; all state and federal whistleblower claims to the maximum extent permitted by law; all claims to any non-vested ownership interest in the Company, contractual or otherwise; and any claim or damage arising out of your employment with and/or separation from the Company (including a claim for retaliation) under any common law theory or any federal, state or local statute or ordinance not expressly referenced above; provided, however, that nothing in this letter agreement shall (i) prevent you from filing a charge with, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or a state fair employment practices agency (except that you acknowledge that you may not recover any monetary benefits in connection with any such claim, charge or proceeding) or (ii) deprive you of any rights you may have to be indemnified by the Company as provided in any agreement between the Company and you or pursuant to the Company's Certificate of Incorporation or by-laws.

3. **Non-Disclosure, Non-Competition and Non-Solicitation** – You acknowledge and reaffirm your obligation to keep confidential and not disclose all non-public information concerning the Company and its clients that you acquired during the course of your employment with the Company, as stated more fully in Section 5 of the Employment Agreement, which remains in full force and effect.

4. **Return of Company Property** – You confirm that you have returned to the Company all keys, files, records (and copies thereof), equipment (including, but not limited to, computer hardware, software and printers, wireless handheld devices, cellular phones, smartphones, tablets, etc.), Company identification, and any other Company-owned property in your possession or control and have left intact all electronic Company documents, including but not limited to those which you developed or helped to develop during your employment. You further confirm that you have cancelled all accounts for your benefit, if any, in the Company's name, including but not limited to, credit cards, telephone charge cards, cellular phone and/or wireless data accounts and computer accounts.

5. **Business Expenses and Final Compensation** – You acknowledge that you have been reimbursed by the Company for all business expenses incurred in conjunction with the performance of your employment and that no other reimbursements are owed to you. You further acknowledge that you have received payment in full for all services rendered in conjunction with your employment by the Company, including payment for all wages, bonuses and accrued, unused vacation time, and that no other compensation is owed to you except as provided herein.

6. **Non-Disparagement** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, for a period of five years following the date hereof you shall not make any false, disparaging or derogatory statements to any person or entity, including any media outlet, regarding the Company or any of its directors, officers, employees, agents or representatives or about the Company's business affairs and financial condition. Further, for a period of five years following the date hereof, neither the Company, nor any of its executive officers or members of its Board will directly or indirectly make, or cause to be made, any false statement, observation or opinion, disparaging your reputation.

7. **Continued Assistance** - You agree that after the Termination Date you will provide all reasonable cooperation to the Company, including but not limited to, assisting the Company transition your job duties, assisting the Company in defending against and/or prosecuting any litigation or threatened litigation, and performing any other tasks as reasonably requested by the Company.

8. **Cooperation** – To the extent permitted by law, you agree to cooperate fully with the Company in the defense or prosecution of any claims or actions which already have been brought, are currently pending, or which may be brought in the future against or on behalf of the Company**,** whether before a state or federal court, any state or federal government agency, or a mediator or arbitrator. Your full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare its claims or defenses, to prepare for trial or discovery or an administrative hearing or a mediation or arbitration and to act as a witness when requested by the Company at reasonable times designated by the Company. You agree that you will notify the Company promptly in the event that you are served with a subpoena or in the event that you are asked to provide a third party with information concerning any actual or potential complaint or claim against the Company.

9. **Amendment and Waiver** – This letter agreement shall be binding upon the parties and may not be modified in any manner, except by an instrument in writing of concurrent or subsequent date signed by duly authorized representatives of the parties hereto. This letter agreement is binding upon and shall inure to the benefit of the parties and their respective agents, assigns, heirs, executors, successors and administrators. No delay or omission by the Company in exercising any right under this letter agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar to or waiver of any right on any other occasion.

10. **Validity** – Should any provision of this letter agreement be declared or be determined by any court of competent jurisdiction to be illegal or invalid, the validity of the remaining parts, terms or provisions shall not be affected thereby and said illegal or invalid part, term or provision shall be deemed not to be a part of this letter agreement.

11. **Confidentiality** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, the terms and contents of this letter agreement, and the contents of the negotiations and discussions resulting in this letter agreement, shall be maintained as confidential by you and your agents and representatives and shall not be disclosed except to the extent required by federal or state law or as otherwise agreed to in writing by the Company.

12. **Nature of Agreement** – You understand and agree that this letter agreement is a severance agreement and does not constitute an admission of liability or wrongdoing on the part of the Company.

13. **Acknowledgments –** You acknowledge that you have been given at least [twenty-one (21) days] to consider this letter agreement, and that the Company advised you to consult with an attorney of your own choosing prior to signing this letter agreement. [You understand that you may revoke this letter agreement for a period of seven (7) days after you sign this letter agreement by notifying me in writing, and the letter agreement shall not be effective or enforceable until the expiration of this seven (7) day revocation period.] You understand and agree that by entering into this agreement, you are waiving any and all rights or claims you might have under the Age Discrimination in Employment Act, as amended by the Older Workers Benefits Protection Act, and that you have received consideration beyond that to which you were previously entitled.

14. **Voluntary Assent** – You affirm that no other promises or agreements of any kind have been made to or with you by any person or entity whatsoever to cause you to sign this letter agreement, and that you fully understand the meaning and intent of this letter agreement. You state and represent that you have had an opportunity to fully discuss and review the terms of this letter agreement with an attorney. You further state and represent that you have carefully read this letter agreement, understand the contents herein, freely and voluntarily assent to all of the terms and conditions hereof and sign your name of your own free act.

15. **Applicable Law** – This letter agreement shall be interpreted and construed by the laws of the State of New Jersey, without regard to conflict of laws provisions. You hereby irrevocably submit to and acknowledge and recognize the jurisdiction of the courts of the State of New Jersey, or if appropriate, a federal court located in the State of New Jersey (which courts, for purposes of this letter agreement, are the only courts of competent jurisdiction), over any suit, action or other proceeding arising out of, under or in connection with this letter agreement or the subject matter hereof.

16. **Entire Agreement** – This letter agreement contains and constitutes the entire understanding and agreement between the parties hereto with respect to your Severance Compensation and the settlement of claims against the Company and cancels all previous oral and written negotiations, agreements and commitments in connection therewith. Nothing in this paragraph, however, shall modify, cancel or supersede your obligations set forth in paragraph 3 herein.

17. **Tax Acknowledgement** – In connection with the payments and consideration provided to you pursuant to this letter agreement, the Company shall withhold and remit to the tax authorities the amounts required under applicable law, and you shall be responsible for all applicable taxes with respect to such payments and consideration under applicable law. You acknowledge that you are not relying upon the advice or representation of the Company with respect to the tax treatment of any of the Severance Compensation set forth in Section 4 of the Employment Agreement.

If you have any questions about the matters covered in this letter agreement, please call me at **[Insert Phone Number]**.

Very truly yours,

By: _____
[Name]
[Title]

I hereby agree to the terms and conditions set forth above. I have been given at least [twenty-one (21) days] to consider this letter agreement and I have chosen to execute this on the date below. I intend that this letter agreement will become a binding agreement between me and the Company [if I do not revoke my acceptance in seven (7) days].

_____ _____
[Insert Employee Name] Date

To be returned to me by **[Return Date – e.g., 21 days from date of receipt of this letter]**.

AMENDMENT TO EMPLOYMENT AGREEMENT

This Amendment (the "Amendment") is made by and between Christine Utter ("Executive") and PTC Therapeutics, Inc. (the "Company") (collectively, "the Parties").

WHEREAS, Executive is employed by PTC pursuant to an Employment Agreement between Executive and PTC dated August 16, 2014 (the "Employment Agreement"); and

WHEREAS, on January 1, 2017 (the "Amendment Effective Date"), in connection with Executive's promotion, the Company is authorized to enter in to an amendment to Executive's Employment Agreement to reflect the title of Senior Vice President, with conforming changes to the Employment Agreement with respect to title, severance compensation, and annual bonus target;

NOW, THEREFORE, for good and valuable consideration, the sufficiency of which is acknowledged hereby, and in consideration of the mutual covenants and undertakings set forth herein, the Parties agree that the following sections of the Employment Agreement are amended and restated as follows as of the Amendment Effective Date:

Section 1(a), Capacity: "Executive shall serve the Company as Senior Vice President, Finance reporting to Shane Kovacs or such other senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Senior Vice President, Finance. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his position as Senior Vice President, Finance as may be reasonably assigned to Executive from time to time by the individual to whom he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080, subject to relocation and Section 2(c)(i) of this Agreement."

Section 2(c), Definition of "Good Reason", clause (ii): "A material adverse change by the Company in Executive's duties, authority or responsibilities as Senior Vice President, Finance of the Company which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business."

Section 3(b), Bonus: In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 40% of Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned."

Section 4, Severance Compensation, subsection (b): "In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth below for twelve (12) months following Executive's termination of employment (the "Severance Period"). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution and nonrevocation of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing 30 days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his Base Salary for the Severance Period, which total amount shall be payable in a lump sum commencing no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) Only if Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his target annual bonus, described in section 3(b) hereof, for the year in which the termination of employment occurs, which total amount shall be payable in a lump sum commencing no later than sixty (60) days following Executive's termination of employment, provided that the Release has been executed and any applicable revocation period has expired as of such date.

(iii) The Company shall continue to provide Executive and his then-enrolled eligible dependents with group health insurance and shall continue to pay the amount of the premium as in effect on the date of such termination for the Severance Period commencing on the effective date of such termination, subject to applicable law and the terms of the respective policies; provided that the Company's obligation to provide the benefits contemplated herein shall terminate upon Executive's becoming eligible for coverage under the medical benefits program of a subsequent employer. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.";

With the following subsections of subsection (4)(b) to be renumbered and referenced consistent with the insertion of the new subsection (4)(b)(ii) set forth above and the renumbering of the prior subsection 4(b)(ii) as subsection 4(b)(iii).

No Other Changes. Except as explicitly provided above, the Parties agree that there are no other changes or amendments to the Employment Agreement and that the Employment Agreement, as amended by this Amendment, remains in full force and effect.

AGREED AND ACCEPTED

PTC Therapeutics, Inc.

By: _/s/ Mark E. Boulding_____

Name: Mark E. Boulding

Title: EVP, Chief Legal Officer

Date: January 17, 2017

AGREED AND ACCEPTED:

Christine Utter

By: _/s/ Christine Utter_____

Date: __January 30, 2017_____

June 1, 2017

Christine Utter
3 Pembroke Court
Marlboro, NJ 07746

Dear Christine,

Congratulations on your offer of promotion to Principal Financial Officer of PTC Therapeutics, Inc. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's success. The effective date of your appointment is the date of your signature below. In this new role you will report to CEO and Founder, Stu Peltz.

Outlined below are details of your compensation following the promotion, subject to your acceptance of this offer:

- Your annual base salary will be increased to $330,000 annually, subject to deductions for taxes and other withholdings as required by law. This represents an increase of 10% over your current base salary.
- Your bonus target will continue to be 40.00% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's annual incentive compensation plan.
- You will receive a one-time grant of 25,000 stock options to purchase shares of common stock of PTC, following a 2 year vesting scheme (50% vesting after one year, and in equal amounts each quarter thereafter).
- You are eligible to receive a one-time appointment bonus of $25,000, payable with acceptance of this letter. Payment will be made as soon as practical taking into account the announcement of the appointment and payroll cycle.

On behalf of PTC, let me again congratulate you on your offer of promotion. We look forward to this next step in your career. Please feel free to contact me if you have any questions concerning your offer of promotion and appointment.

Sincerely, Accepted by:

/s/ Stuart Peltz /s/ Christine Utter_ Date: 6/2/17_
Stuart Peltz, CEO Christine Utter

cc: Martin Rexroad, SVP, Human Resources

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, 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: August 9, 2017

By: /s/ STUART W. PELTZ

Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Christine Utter, 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: August 9, 2017

By: /s/ CHRISTINE UTTER

Christine Utter
Principal Financial Officer
(Principal Financial and Accounting 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 June 30, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, 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: August 9, 2017

By: /s/ STUART W. PELTZ

Stuart W. Peltz
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 June 30, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Christine Utter, Principal Financial 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: August 9, 2017

By: /s/ CHRISTINE UTTER

Christine Utter
Principal Financial Officer
(Principal Financial and Accounting Officer)