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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 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 October 31, 2017, there were 41,489,580 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- our 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, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act and through the grant of pediatric exclusivity;

- 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 Complete Response letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, either via the outcome of any formal dispute resolution request or other interactions with the FDA, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments 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 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 labeling 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 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;
- the impact of litigation that has been brought against us;
- 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)

	September 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 141,838	$ 58,321
Marketable securities	27,472	173,345
Trade receivables, net	38,744	24,929
Inventory	7,792	—
Prepaid expenses and other current assets	5,413	4,691
Total current assets	221,259	261,286
Fixed assets, net	6,882	7,429
Intangible assets, net	138,422	—
Deposits and other assets	1,157	630
Total assets	$ 367,720	$ 269,345
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 64,054	$ 48,759
Deferred revenue	6,122	—
Other current liabilities	1,723	865
Total current liabilities	71,899	49,624
Deferred revenue - long-term	6,579	1,587
Long-term debt	143,091	98,216
Other long-term liabilities	269	335
Total liabilities	221,838	149,762
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 41,463,121 shares at September 30, 2017. Authorized 125,000,000 shares; issued and outstanding 34,169,410 shares at December 31, 2016	41	34
Additional paid-in capital	958,206	856,142
Accumulated other comprehensive income (loss)	3,013	(1,485)
Accumulated deficit	(815,378)	(735,108)
Total stockholders' equity	145,882	119,583
Total liabilities and stockholders' equity	$ 367,720	$ 269,345

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Revenues:				
Net product revenue	$ 41,780	$ 22,013	$ 116,113	$ 56,328
Collaboration and grant revenue	73	973	249	1,186
Total revenues	41,853	22,986	116,362	57,514
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible asset	1,582	—	2,142	—
Amortization of acquired intangible asset	9,716	—	9,952	—
Research and development	30,024	31,396	88,222	91,622
Selling, general and administrative	31,423	23,654	85,788	72,958
Total operating expenses	72,745	55,050	186,104	164,580
Loss from operations	(30,892)	(32,064)	(69,742)	(107,066)
Interest expense, net	(3,421)	(2,133)	(8,648)	(6,149)
Other income (expense), net	766	(786)	(1,373)	(1,893)
Loss before income tax expense	(33,547)	(34,983)	(79,763)	(115,108)
Income tax expense	(191)	(184)	(507)	(206)
Net loss attributable to common stockholders	$ (33,738)	$ (35,167)	$ (80,270)	$ (115,314)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	41,296,740	34,088,741	38,433,749	34,002,952
Net loss per share—basic and diluted (in dollars per share)	$ (0.82)	$ (1.03)	$ (2.09)	$ (3.39)

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Net loss	$ (33,738)	$ (35,167)	$ (80,270)	$ (115,314)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities, net of tax	31	(189)	—	429
Foreign currency translation gain	983	60	4,498	1,527
Comprehensive loss	$ (32,724)	$ (35,296)	$ (75,772)	$ (113,358)

See accompanying unaudited notes.

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

	Nine Months Ended September 30,	
	2017	**2016**
Cash flows from operating activities		
Net loss	$ (80,270)	$ (115,314)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	11,743	2,477
Change in valuation of warrant liability	3	44
Non-cash interest expense	4,999	4,487
Loss on disposal of asset	5	—
Amortization of premiums on investments	493	1,610
Amortization of debt issuance costs	308	224
Share-based compensation expense	24,082	26,610
Benefit for deferred income taxes	—	(222)
Unrealized foreign currency transaction (gains) losses, net	(364)	1,401
Changes in operating assets and liabilities:		
Inventory, net	(3,625)	—
Prepaid expenses and other current assets	(570)	1,095
Trade receivables, net	(10,994)	(16,035)
Deposits and other assets	(485)	(154)
Accounts payable and accrued expenses	11,807	2,080
Other liabilities	807	682
Deferred revenue	10,710	768
Net cash used in operating activities	(31,351)	(90,247)
Cash flows from investing activities		
Purchases of fixed assets	(1,058)	(540)
Purchases of marketable securities	(19,467)	(73,692)
Sale and redemption of marketable securities	164,847	155,582
Acquisition, including transaction costs	(77,163)	—
Net cash provided by investing activities	67,159	81,350
Cash flows from financing activities		
Proceeds from exercise of options	1,437	926
Proceeds from shares issued under employee stock purchase plan	1,362	—
Debt issuance costs related to secured term loan	(432)	—
Proceeds from issuance of secured term loan	40,000	—
Net cash provided by financing activities	42,367	926
Effect of exchange rate changes on cash	5,342	235
Net increase (decrease) in cash and cash equivalents	83,517	(7,736)
Cash and cash equivalents, beginning of period	58,321	58,022
Cash and cash equivalents, end of period	$ 141,838	$ 50,286
Supplemental disclosure of cash information		
Cash paid for interest	$ 5,496	$ 4,513
Cash paid for income taxes	$ 616	$ 633
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized gain on marketable securities, net of tax	$ —	$ 429

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
September 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), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. 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 aged 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 DMD in patients five years and older.

The Company’s marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, 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 September 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"), for which the FDA granted a standard review. In October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it is unable to approve the application in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. In response, the Company has filed a formal dispute resolution request (FDRR) with the Office of New Drugs of the FDA, which, as per FDA draft guidelines, would typically involve a time-frame of one-to-two months to receive a response from the FDA. The FDA's complete response letter also mentioned other nonclinical and CMC matters that the Company is in the process of addressing. 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 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 clinical development of Translarna for nmCF and has closed the 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 September 30, 2017, the Company had an accumulated deficit of approximately $815.4 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 nine month period ended September 30, 2017 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of September 30, 2017 and for the three and nine months ended September 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 September 30, 2017 and for the three and nine months ended September 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 nine month periods ended September 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 (see 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 Transaction 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 Transaction. 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:

	September 30, 2017	December 31, 2016
Raw materials	$ 182	$ —
Work in progress	2,715	—
Finished goods	4,895	—
Total inventory	$ 7,792	$ —

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, 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 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 $0.8 million as of September 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 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 has elected to use the modified retrospective approach (retrospective application with the cumulative effect of applying the updated standard recognized at the date of initial application and providing certain additional disclosures) 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. The Company expects the adoption of the new revenue standard to have an impact on its financial reporting disclosures and internal controls over financial reporting. The Company has established 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 are provided with full valuation allowance as of September 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 September 30, 2017 and December 31, 2016:

	September 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	$ 27,472	$ —	$ 27,472	$ —
Warrant liability	$ 4	$ —	$ —	$ 4
Stock appreciation rights liability	$ 1,723	$ —	$ —	$ 1,723

	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 September 30, 2017 and December 31, 2016.

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

	September 30, 2017			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
Commercial paper	$ —	$ —	$ —	$ —
Corporate debt securities	27,675	2	(205)	27,472
Government obligations	—	—	—	—
	$ 27,675	$ 2	$ (205)	$ 27,472

	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 September 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 September 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 September 30, 2017 and December 31, 2016 mature as follows:

	September 30, 2017	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ —	$ —
Corporate debt securities	27,472	—
Government obligations	—	—
Total Marketable securities	$ 27,472	$ —

	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 September 30, 2017:

	Level 3 liabilities	
	Warrants	SARs
Beginning balance as of December 31, 2016	$ 1	$ 865
Change in fair value	3	1,922
Payments	—	(1,064)
Ending balance as of September 30, 2017	$ 4	$ 1,723

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 September 30, 2017 include (i) volatility (70%), (ii) risk free interest rate (1.47%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($20.01), and (v) expected life (1.9—2.0 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 September 30, 2017 include (i) volatility (63%—71%), (ii) risk free interest rate (1.06%—1.55%), (iii) strike price ($6.76-$30.86), (iv) fair value of

common stock ($20.01), and (v) expected life (0.3—2.3 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 nine months ended September 30, 2017:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at June 30, 2017	$ (234)	$ 2,233	$ 1,999
Other comprehensive income before reclassifications	31	983	1,014
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income	31	983	1,014
Balance at September 30, 2017	$ (203)	$ 3,216	$ 3,013

	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 income before reclassifications	—	4,498	4,498
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income	—	4,498	4,498
Balance at September 30, 2017	$ (203)	$ 3,216	$ 3,013

5. Accounts payable and accrued expenses

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

	September 30, 2017	December 31, 2016
Employee compensation, benefits, and related accruals	$ 12,915	$ 13,649
Consulting and contracted research	9,536	11,505
Professional fees	1,873	1,237
Sales allowance and other costs	29,190	13,245
Accounts payable	5,994	6,298
Other	4,546	2,825
	$ 64,054	$ 48,759

6. Warrants

All of the Company's outstanding warrants were classified as liabilities as of September 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 September 30, 2017 and December 31, 2016:

	September 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 September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Numerator				
Net loss	$ (33,738)	$ (35,167)	$ (80,270)	$ (115,314)
Denominator				
Denominator for basic and diluted net loss per share	41,296,740	34,088,741	38,433,749	34,002,952
Net loss per share:				
Basic and diluted	$ (0.82) *	$ (1.03) *	$ (2.09) *	$ (3.39) *

*In the three and nine months ended September 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 September 30,	
	2017	2016
Stock Options	6,612,765	5,832,166
Unvested restricted stock awards and units	402,853	272,579
Total	7,015,618	6,104,745

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 September 30, 2017, awards for 823,506 shares of common stock are available for issuance.

From January 1, 2017 through September 30, 2017, the Company issued a total of 1,809,873 stock options to various employees. Of those, 541,550 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	1,809,873	$ 12.12		
Exercised	(132,795)	$ 10.82		
Forfeited/Cancelled	(918,629)	$ 33.20		
Outstanding at September 30, 2017	6,612,765	$ 29.21	7.43 years	$ 22,786
Vested or Expected to vest at September 30, 2017	2,719,750	$ 24.50	8.59 years	$ 11,858
Exercisable at September 30, 2017	3,718,713	$ 33.07	6.51 years	$ 10,014

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

	Nine months ended September 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 nine-month period ended September 30, 2017 was $8.30 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	363,194	$ 11.64
Vested	(180,861)	$ 14.19
Forfeited	(51,131)	$ 13.90
Unvested at September 30, 2017	402,853	$ 15.62

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 September 30, 2017, a total of 213,197 SARs vested and the Company recorded $1.9 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 September 30, 2017, the Company issued 191,787 shares of common stock and recorded $0.6 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 September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Research and development	$ 3,624	$ 4,319	$ 11,986	$ 12,734
Selling, general and administrative	3,544	4,640	12,096	13,876
Total	$ 7,168	$ 8,959	$ 24,082	$ 26,610

As of September 30, 2017, there was approximately $45.6 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 2.05 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 September 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	**September 30, 2017**	**December 31, 2016**
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(2,208)	(2,457)
Less: Debt discount, net(1)	(44,329)	(49,327)
Net carrying amount	$ 103,463	$ 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 $120.3 million as of September 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 September 30, 2017, the remaining contractual life of the Convertible Notes is approximately 4.9 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Contractual interest expense	$ 1,134	$ 1,131	$ 3,375	$ 3,372
Amortization of debt issuance costs	86	77	249	224
Amortization of debt discount	1,725	1,546	4,999	4,487
Total	$ 2,945	$ 2,754	$ 8,623	$ 8,083
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.

The Company is currently involved in various legal proceedings (refer to Item 1. Legal Proceedings for further details on the lawsuits filed). The Company denies any allegations of wrongdoing and intends to vigorously defend against these lawsuits. The Company is unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of this matter in a manner adverse to the Company and for which it incurs substantial costs or damages not covered by the Company's directors’ and officers’ liability insurance would have a material adverse effect on its financial condition and business. In addition, the litigation could adversely impact the Company's reputation and divert management’s attention and resources from other priorities, including the execution of business plans and strategies that are important to the Company's ability to grow its business, any of which could have a material adverse effect on the Company's business.

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 over its expected useful life of approximately seven years. The Company utilized an economic use method approach for recording the amortization in the second quarter. Given the inherent uncertainty of the Company's sales projections, the Company concluded that amortizing the asset on a straight line basis is a more appropriate method. Had amortization been recorded on a straight line basis since the acquisition, there would have been an additional $4.3 million of amortization recorded in the three months ended June 30, 2017. This amount is immaterial to the financial statements and is recorded in the three months ended September 30, 2017.

As of September 30, 2017, the Company recognized accumulated amortization of $10.0 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 September 30, 2017
2017 (1)	$	5,428
2018		21,713
2019		21,713
2020		21,713
2021 and thereafter		67,854
Total	$	138,421

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

12. Subsequent events

In October 2017, the Company announced that the Sunfish, a two-part clinical trial in pediatric and adult type 2 and type 3 spinal muscular atrophy initiated in the fourth quarter of 2016, had transitioned into the pivotal second part of its study. The achievement of this milestone triggered a $20.0 million payment to the Company from F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which was recorded as collaboration revenue at time of achievement.

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 September 30, 2017, we recognized $32.0 million in sales of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, and $9.8 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

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 2017, the Office of Drug Evaluation I of the U.S. Food and Drug Administration, or FDA, issued a complete response letter for our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, stating that it is unable to approve the NDA in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. In response, we have filed a formal dispute resolution request (FDRR) with the Office of New Drugs of the FDA, which, as per FDA draft guidelines, would typically involve a time-frame of one-to-two months to receive a response from the FDA. The letter also mentioned other nonclinical and CMC matters, which we are in the process of addressing. The letter was in response to our filing of our Translarna NDA via the FDA's file over protest regulations during the first quarter of 2017, for which the FDA granted a standard review. Previously, 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 Translarna NDA.

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 dispute resolution 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 European Medicines Agency, or 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 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). We have submitted to 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 includes 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 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 EEA and other jurisdictions would prevent us from marketing our products in such regions.*"

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, we have 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. We are committed to make this important therapy available to all eligible patients in the United States and to study its long-term benefits.

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. 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. Additionally, we expect to be granted pediatric exclusivity, which 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. 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 1,500 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.

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 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 trial was consistent with previous studies and no new safety signals were identified. Based on the results of ACT CF, we have discontinued our clinical development of Translarna for nmCF and have closed our 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 have completed enrollment for our aniridia study and we anticipate results during 2018. In the third quarter of 2017 we stopped enrollment and began to wind down 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. Additionally, an interim analysis of the five cohorts in Part 1 of the Sunfish study treated with RG7916 for 28 days or longer demonstrated an exposure-dependent increase in SMN protein. 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. In October 2017, Sunfish transitioned into the pivotal second part of its study, which triggered a $20 million milestone payment to us from Roche. We anticipate that Firefish will move into the pivotal second part of its study in the coming few months. 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 September 30, 2017, we had an accumulated deficit of $815.4 million. We had a net loss of $80.3 million and $115.3 million for the nine months ended September 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 current and former executive officers and the derivative lawsuits brought against us, as a nominal defendant, certain of our current and former executive officers and certain of our current and former directors, 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 SMA with Roche and the SMA Foundation (SMAF) had 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.

In October 2017, we announced that the Sunfish study had transitioned into the pivotal second part of its study. The achievement of this milestone triggered a $20.0 million payment to us from Roche which we recorded as collaboration revenue at time of achievement.

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 nine months ended September 30, 2017 and 2016.

	Three Months Ended September 30,	
	2017	2016
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 20,834	$ 22,088
Cancer stem cell	555	1,689
Next generation nonsense readthrough	1,365	1,621
EMFLAZA	1,859	—
Other research and preclinical	5,411	5,998
Total research and development	$ 30,024	$ 31,396

	Nine Months Ended September 30, 2017	
	2017	2016
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 61,276	$ 66,133
Cancer stem cell	2,735	5,523
Next generation nonsense readthrough	4,145	5,577
EMFLAZA	4,303	—
Other research and preclinical	15,763	14,389
Total research and development	$ 88,222	$ 91,622

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 and miscellaneous selling costs.

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 Agreement.

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 September 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:

	September 30, 2017	December 31, 2016
Raw materials	$ 182	$ —
Work in progress	2,715	—
Finished goods	4,895	—
Total inventory	$ 7,792	$ —

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, 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 September 30, 2017, we issued a total of 1,809,873 stock options to various employees. Of those, 541,550 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 nine months ended September 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 nine month period ended September 30, 2017 was $8.30 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	363,194	$ 11.64
Vested	(180,861)	$ 14.19
Forfeited	(51,131)	$ 13.90
September 30, 2017	402,853	$ 15.62

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 September 30, 2017, a total of 213,197 SARs vested and we recorded $1.9 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 September 30, 2017, we issued 191,787 shares of common stock and recorded $0.6 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 September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
Research and development	$ 3,624	$ 4,319	$ 11,986	$ 12,734
Selling, general and administrative	3,544	4,640	12,096	13,876
Total	$ 7,168	$ 8,959	$ 24,082	$ 26,610

As of September 30, 2017, there was approximately $45.6 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 2.05 years.

Results of operations

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

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

(in thousands)	Three Months Ended September 30, 2017	2016	Change 2017 vs. 2016
Net product revenue	$ 41,780	$ 22,013	$ 19,767
Collaboration and grant revenue	73	973	(900)
Cost of product sales, excluding amortization of acquired intangible asset	1,582	—	1,582
Amortization of acquired intangible asset	9,716	—	9,716
Research and development expense	30,024	31,396	(1,372)
Selling, general and administrative expense	31,423	23,654	7,769
Interest expense, net	(3,421)	(2,133)	(1,288)
Other income (expense), net	766	(786)	1,552
Income tax expense	(191)	(184)	(7)

Net product revenues. Net product revenues were $41.8 million for the three months ended September 30, 2017, an increase of $19.8 million, or 90%, from $22.0 million for the three months ended September 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 September 30, 2017 and $1.0 million for the three months ended September 30, 2016. Revenues are primarily from ongoing collaboration arrangements with Roche.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales were $1.6 million for the three months ended September 30, 2017. Cost of product sales consist primarily of 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.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $9.7 million for the three months ended September 30, 2017 resulting from the acquisition of EMFLAZA. The amount allocated to the EMFLAZA intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to EMFLAZA, the period of estimated future cash flows.

Research and development expense. Research and development expense was $30.0 million for the three months ended September 30, 2017, a decrease of $1.4 million, or 4%, from $31.4 million for the three months ended September 30, 2016. The decrease resulted primarily due to to the completion of our Phase 3 Translarna trials at the end of 2016, partially offset by start-up clinical activities and regulatory spend.

Selling, general and administrative expense. Selling, general and administrative expense was $31.4 million for the three months ended September 30, 2017, an increase of $7.8 million, or 33%, from $23.7 million for the three months ended September 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 $3.4 million for the three months ended September 30, 2017, an increase of $1.3 million, or 60%, from $2.1 million for the three months ended September 30, 2016. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Agreement in addition to lower interest income from investments.

Other income (expense), net. Other income, net was $0.8 million for the three months ended September 30, 2017, a decrease in expense of $1.6 million, or 197%, from other expense, net of $0.8 million for the three months ended September 30, 2016. The change from expense to income resulted primarily from exchange rate changes in the current period.

Income tax expense. Income tax expense was $0.2 million for the three months ended September 30, 2017 and $0.2 million for the three months ended September 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 September 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.

Nine months ended September 30, 2017 compared to nine months ended September 30, 2016

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

(in thousands)	Nine Months Ended September 30, 2017	Nine Months Ended September 30, 2016	Change 2017 vs. 2016
Net product revenue	$ 116,113	$ 56,328	$ 59,785
Collaboration and grant revenue	249	1,186	(937)
Cost of product sales, excluding amortization of acquired intangible asset	2,142	—	2,142
Amortization of acquired intangible asset	9,952	—	9,952
Research and development expense	88,222	91,622	(3,400)
Selling, general and administrative expense	85,788	72,958	12,830
Interest expense, net	(8,648)	(6,149)	(2,499)
Other expense, net	(1,373)	(1,893)	520
Income tax expense	(507)	(206)	(301)

Net product revenues. Net product revenues were $116.1 million for the nine months ended September 30, 2017, an increase of $59.8 million, or 106%, from $56.3 million for the nine months ended September 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 nine months ended September 30, 2017, a decrease of $0.9 million, or 79%, from $1.2 million for the nine months ended September 30, 2016. These revenues are primarily from ongoing collaboration arrangements with Roche.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales were $2.1 million for the nine months ended September 30, 2017. Cost of product sales consist primarily of 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.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $10.0 million for the nine months ended September 30, 2017 resulting from the acquisition of EMFLAZA. The amount allocated to the EMFLAZA intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to EMFLAZA, the period of estimated future cash flows.

Research and development expense. Research and development expense was $88.2 million for the nine months ended September 30, 2017, a decrease of $3.4 million, or 4%, from $91.6 million for the nine months ended September 30, 2016. The decrease resulted primarily from the completion of our Phase 3 Translarna trials at the end of 2016 partially offset by start-up clinical activities and regulatory spend.

Selling, general and administrative expense. Selling, general and administrative expense was $85.8 million for the nine months ended September 30, 2017, an increase of $12.8 million, or 18%, from $73.0 million for the nine months ended September 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 $8.6 million for the nine months ended September 30, 2017, an increase in expense of $2.5 million, or 41%, from interest expense of $6.1 million for the nine months ended September 30, 2016. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Agreement partially offset by interest income from investments.

Other expense, net. Other expense, net was $1.4 million for the nine months ended September 30, 2017, a decrease in expense of $0.5 million, or 27% , from other expense, net of $1.9 million for nine months ended September 30, 2016. The decrease resulted primarily from foreign currency gains due to changes in exchange rates in the current period.

Income tax expense. Income tax expense was $0.5 million for the nine months ended September 30, 2017 and $0.2 million for the nine months ended September 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 nine months ended September 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 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 September 30, 2017, we had cash, cash equivalents and marketable securities of $169.3 million.

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

	Nine Months Ended September 30,	
(in thousands)	**2017**	**2016**
Cash provided by (used in):		
Operating activities	(31,351)	(90,247)
Investing activities	67,159	81,350
Financing activities	42,367	926

Net cash used in operating activities was $31.4 million for the nine months ended September 30, 2017 and $90.2 million for the nine months ended September 30, 2016. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities, partially offset by increased cash receipts resulting from higher net product revenues.

Net cash provided by investing activities was $67.2 million for the nine months ended September 30, 2017 and $81.4 million for the nine months ended September 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 $42.4 million for the nine months ended September 30, 2017 and $0.9 million for the nine months ended September 30, 2016. Cash provided by financing activities in the current period is primarily attributable to entry into the Credit Agreement 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 trials, non-clinical studies or CMC assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA 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 formal dispute resolution request process with the FDA, or otherwise, and including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA 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 current and former executive officers and the derivative lawsuits brought against us, as a nominal defendant, certain of our current and former executive officers and certain of our current and former directors, 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 September 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)	$ 10,232	$ 1,082	$ 3,394	$ 3,542	$ 2,214
Credit agreement, including interest (2)	47,616	2,900	31,101	13,615	—
Total contractual obligations	$ 57,848	$ 3,982	$ 34,495	$ 17,157	$ 2,214

(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 September 30, 2017.

(2) Under the terms of the Credit Agreement, 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 September 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 September 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 September 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 September 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 former Chief Financial Officer. The lawsuits have been consolidated into one action captioned *In re PTC Therapeutics, Inc. Securities Litigation*, No. 16-1224 (KM) (the "Securities Class Action"). A consolidated amended complaint was filed on January 13, 2017. The complaint 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. On February 14, 2017, the defendants filed a motion to dismiss the consolidated amended complaint. On August 28, 2017, the motion to dismiss was granted in part and denied in part. On September 25, 2017, defendants filed an answer and affirmative defenses to the consolidated amended complaint.

On September 19, 2017, a purported stockholder of the Company filed a derivative lawsuit in the United States District Court for the District of New Jersey against our Chief Executive Officer, our former Chief Financial Officer, and current or former directors (Michael Schmertzler; Richard Aldrich; Allan Jacobson; Adam Koppel; Michael Kranda; C. Geoffrey McDonough; Ronald C. Renaud, Jr.; David P. Southwell; Jerome Zeldis; and Glenn D. Steele, Jr.), with the caption *Choi v. Peltz, et al.*, No. 17-cv-07216. The Company is named as a nominal defendant. On October 10, 2017, another purported stockholder of the Company filed a derivative lawsuit in the United States District Court for the District of New Jersey against the same defendants and nominal defendant, with the caption *Kim v. Peltz, et al.*, No. 17-cv-08062 (together with the *Choi* action, the "Derivative Actions"). The Derivative Actions allege violations of Section 14(a) of the Securities Exchange Act of 1934, breaches of defendants' fiduciary duties, unjust enrichment, abuse of control, and gross mismanagement based on allegations that defendants made or approved improper statements regarding the NDA for Translarna for the treatment of nmDMD that the Company submitted to the FDA in December 2015. The Derivative Actions seek, among other things, any damages sustained by the Company as a result of the defendants' alleged wrongdoing (including fees associated with the Securities Class Action), an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures, restitution from the defendants, and 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 have 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 have 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, 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.

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.

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 September 30, 2017, we had an accumulated deficit of $815.4 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 through 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 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 formal dispute resolution request 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 formal dispute resolution request process or otherwise, and by determining to file our formal dispute resolution request, 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 "Risks Related to the Regulation of our Products and our Product Candidates" "*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 and security agreement with MidCap Financial Trust, 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 trials, non-clinical studies or CMC assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA 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 current and former executive officers and the derivative lawsuits brought against us, as a nominal defendant, certain of our current and former executive officers and certain of our current and former directors, 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 formal dispute resolution request process with the FDA or otherwise, and including, if required, performing additional clinical trials, non-clinical studies or CMC assessments 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 Trust;
- 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 formal dispute resolution request process with the FDA, or otherwise, and including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA 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 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 have 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 have discontinued our 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 the U.S., the anticipated joint framework for tax reform from President Trump's Administration and Congressional tax writing committees was released in September 2017. Entitled a "Unified framework for fixing our broken tax code", this document outlines the framework upon which a potential U.S. tax reform is expected to be based, which proposals include, among other items, a significant reduction to the U.S. corporate tax rate and the introduction of a territorial tax regime.

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. In October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it is unable to approve the application in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA, which, as per FDA draft guidelines, would typically involve a time-frame of one-to-two months to receive a response from the FDA. The FDA's complete response letter also mentioned other nonclinical and CMC matters that we are in the process of addressing.

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 formal dispute resolution request 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 complete response 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 trials, non-clinical studies or CMC assessments or analyses at significant cost. Even if we are able to enroll and fund any such additional trials or studies or complete such assessments or analyses, there is substantial risk that the results would not ultimately support the approval of the current NDA 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 age 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 have 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 complete response letter to our NDA 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;
- 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 formal dispute resolution request process with the FDA or otherwise, and including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA 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 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. As a result, we discontinued our 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 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. The FDA reiterated its view in the complete response letter that it sent to us in October 2017.

Although we filed a formal dispute resolution request for our NDA with the Office of New Drugs of 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 formal dispute resolution request 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 its Complete Response 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 trials, non-clinical studies or CMC assessments or analyses at significant cost. Even if we are able to enroll and fund any such additional trials or studies or complete such assessments or analyses, there is substantial risk that the results would not ultimately support the approval of the current NDA 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 trial for Translarna in nmCF. As a result, we have discontinued our 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 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 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 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 stopped 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. As a result, we have discontinued our 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 payors 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 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.

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). We have submitted to 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 includes data from this study. However, there can be no assurances that we will successfully obtain such label extension.

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 formal dispute resolution request process or otherwise, and by determining to file a dispute resolution request for our NDA , 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.

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. In October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it is unable to approve the application in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA, which, as per FDA draft guidelines, would typically involve a time-frame of one-to-two months to receive a response from the FDA. The letter also mentioned other nonclinical and CMC matters that we are in the process of addressing.

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 formal dispute resolution request process or otherwise, we will continue to be unable to resolve the matters raised by the FDA in its complete response 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 trials, non-clinical studies or CMC assessments or analyses at significant cost. Even if we are able to enroll and fund any such additional trials or studies or complete such assessments or analyses, there is substantial risk that the results would not ultimately support the approval of the current NDA 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, in July 2016 we filed a formal dispute resolution request with the Office of New Drugs of 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 formal dispute resolution request procedure. In its 2016 Refuse to File letter and in its 2017 complete response 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 escalating our appeal to the next supervisory level of the FDA, we have postponed other strategic pathways such as commencing direct litigation or discussing the design of a new clinical trial with the FDA. Such alternative strategies may be more effective than the formal dispute resolution request process 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 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 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 received such a request from the FDA and we are in the process of discussing with the FDA what the nature and objective of the required studies needed to obtain such data will be. There is no guarantee that we will be able to initiate the necessary study, and to the extent that a study is initiated, there is no guarantee of its completion and submission to the FDA, which would cause the additional six-month exclusivity to be granted.

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 payors 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 products 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 formal dispute resolution request process with the FDA, or otherwise, and including whether we will be required to complete any additional clinical trials, non-clinical studies or CMC assessments 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 a securities class action lawsuit initiated against us and certain of our current and former executive officers during the first quarter of 2016 and derivative lawsuits brought against us, as a nominal defendant, certain of our current and former executive officers and certain of our current and former directors during the third quarter of 2017. 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 September 30, 2017, we issued options to purchase an aggregate of 61,000 shares of common stock to certain new hire employees at a weighted-average exercise price of $20.02 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.

Exhibit Number	Description of Exhibit
10.1+	Consulting Services Agreement between the Registrant and Mark A. Rothera (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on August 8, 2017)
10.2+	Consulting Services Agreement between the Registrant and Dr. C. Geoffrey McDonough (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on September 18, 2017)
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.

+ Management contract, compensatory plan or arrangement.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: November 2, 2017

By: /s/ Christine Utter

Christine Utter

Principal Financial Officer

(Principal Financial and Accounting Officer and Duly Authorized Signatory)

EXHIBIT INDEX

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+ 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 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: November 2, 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: November 2, 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 September 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: November 2, 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 September 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: November 2, 2017

By: /s/ CHRISTINE UTTER

Christine Utter
Principal Financial Officer
(Principal Financial and Accounting Officer)