UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2015

OR

o 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 x No o

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 x No o

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

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

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

As of November 4, 2015 there were 34,271,694 shares of Common Stock, $0.001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form10-Q contains forward looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form10-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:

- the timing of PTC's planned regulatory filings with respect to the results of our Phase 3 confirmatory trial in nonsense mutation Duchenne muscular dystrophy, or nmDMD, including the completion of our new drug application, or NDA, with the U.S. Food and Drug Administration, or FDA, our submission of the clinical trials results with the European Medicines Agency, or EMA, and other filings with regulatory bodies outside of the United States and European Economic Area, or EEA;
- the timing and conduct of our clinical trials and studies of Translarna™ (ataluren) for the treatment of cystic fibrosis, mucopolysaccharidosis type I, or MPS I, and aniridia, caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our cancer stem cell program, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- the rate and degree of market acceptance and clinical utility of Translarna;
- our ability to commercialize Translarna in general, and specifically as a treatment for nmDMD, including the timing of such commercialization and our ability to successfully negotiate adequate pricing and reimbursement processes on a timely basis, or at all, in the countries in which we may obtain regulatory approval, including the countries in the European Economic Area;
- the timing of and our ability to obtain additional marketing authorizations for Translarna and our other product candidates, and the ability of Translarna and our other product candidates to meet existing or future regulatory standards;
- 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;
- our estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and our ability to identify such patients;
- our ability to expand the approved product label of Translarna for the treatment of nmDMD;
- the timing and scope of our commercial infrastructure expansion, including the growth of our international presence in Europe and in other territories;
- the potential receipt of revenues from future sales of Translarna and other product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third party manufacturers to manufacture and deliver Translarna in commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy its other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of Translarna and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our plans to pursue development of Translarna for additional indications other than nmDMD and cystic fibrosis, MPS I, and aniridia, caused by nonsense mutations;
- our ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is

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conditioned upon, among other things, submission of the final report, including additional efficacy and safety data from our Phase 3 confirmatory trial in nmDMD during 2015 and which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization;

- our ability to advance our earlier stage programs, including our antibacterial program;
- our plans to pursue research and development of other product candidates;
- the potential advantages of Translarna;
- our estimates regarding expenses, future revenues, 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;
- our intellectual property position;
- the impact of government laws and regulations;
- our competitive position; and
- our expectations with respect to the development and regulatory status of our product candidate 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, 2014 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,” “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.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.
Consolidated Balance Sheets (unaudited)
In thousands (except per share data)

	September 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 140,712	$ 49,748
Marketable securities	230,809	265,493
Prepaid expenses and other current assets	4,627	3,885
Receivables, net	8,016	4,445
Total current assets	384,164	323,571
Fixed assets, net	8,799	9,159
Deposits and other assets	3,137	489
Total assets	$ 396,100	$ 333,219
Liabilities and stockholders’ equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 31,695	$ 29,121
Deferred revenue	—	3,354
Total current liabilities	31,695	32,475
Long-term debt	93,198	—
Other long-term liabilities	2,056	2,277
Total liabilities	126,949	34,752

Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 33,915,059 shares at September 30, 2015. Authorized 125,000,000 shares; issued and outstanding 32,898,392 shares at December 31, 2014	34	33
Additional paid-in capital	812,294	721,722
Accumulated other comprehensive loss	(1,127)	(737)
Accumulated deficit	(542,050)	(422,551)
Total stockholders' equity	269,151	298,467
Total liabilities and stockholders' equity	$ 396,100	$ 333,219

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Revenues:				
Net product revenue	$ 9,772	$ 81	$ 21,002	$ 81
Collaboration revenue	2	716	547	11,280
Grant revenue	2	897	2,483	1,226
Total revenues	9,776	1,694	24,032	12,587
Operating expenses:				
Research and development	30,640	18,765	86,768	52,967
Selling, general and administrative	21,368	10,530	56,193	26,803
Total operating expenses	52,008	29,295	142,961	79,770
Loss from operations	(42,232)	(27,601)	(118,929)	(67,183)
Interest (expense)/income, net	(852)	354	170	774
Other expense, net	(51)	(35)	(507)	(75)
Loss before income tax expense	(43,135)	(27,282)	(119,266)	(66,484)
Income tax expense	(88)	—	(233)	—
Net loss	$ (43,223)	$ (27,282)	$ (119,499)	$ (66,484)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	33,908,853	29,351,693	33,528,833	28,441,827
Net loss per share—basic and diluted (in dollars per share)	$ (1.27)	$ (0.93)	$ (3.56)	$ (2.34)

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Net loss	$ (43,223)	$ (27,282)	$ (119,499)	$ (66,484)
Other comprehensive loss:				
Unrealized loss on marketable securities	(483)	(159)	(582)	(139)
Foreign currency translation (loss)/ gain	(149)	—	192	—
Comprehensive loss	$ (43,855)	$ (27,441)	$ (119,889)	$ (66,623)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Consolidated Statements of cash flows (unaudited)
In thousands

	Nine months ended September 30,	
	2015	2014
Cash flows from operating activities		
Net loss	$ (119,499)	$ (66,484)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	2,079	1,693
Change in valuation of warrant liability	(152)	78
Non-cash interest expense	736	—
Amortization of debt issuance costs	37	—
Amortization of premiums on investments	1,319	1,225
Share-based compensation expense	26,130	12,605
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(748)	(1,232)
Receivables	(3,582)	(338)
Deposits and other assets	183	(937)
Accounts payable and accrued expenses	2,871	4,286
Other long-term liabilities	(69)	(68)
Deferred revenue	(3,310)	(109)
Net cash used in operating activities	(94,005)	(49,281)
Cash flows from investing activities		
Purchases of fixed assets	(1,764)	(1,156)
Purchases of marketable securities	(134,299)	(132,602)
Sales & redemptions of marketable securities	167,082	101,775
Net cash provided by/(used in) investing activities	31,019	(31,983)
Cash flows from financing activities		
Payments on long-term debt	—	(49)
Proceeds from exercise of options	8,689	389
Net proceeds from public offerings	—	118,383
Debt issuance costs related to convertible notes	(4,650)	—
Proceeds from issuance of convertible notes	150,000	—
Net cash provided by financing activities	154,039	118,723
Effect of exchange rate changes on cash	(89)	—
Net increase in cash and cash equivalents	90,964	37,459
Cash and cash equivalents, beginning of period	49,748	15,414
Cash and cash equivalents, end of period	$ 140,712	$ 52,873
Supplemental disclosure of cash information		
Cash paid for interest	$ —	$ 1
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized loss on marketable securities	$ (582)	$ (139)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
September 30, 2015
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 orally administered, small molecule therapeutics targeting an area of RNA biology referred to as post-transcriptional control. The letters “PTC” in the corporate name are an acronym for post-transcriptional control processes, which are the regulatory events that occur in cells during and after a messenger RNA is copied from DNA through the transcription process. The Company has discovered all of its compounds currently under development using its proprietary technologies. The Company plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. The Company believes that systematically targeting post-transcriptional control processes represents an unexploited approach to drug discovery and development. The Company’s internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases.

The Company’s lead product, Translarna™ (ataluren) received marketing authorization from the European Commission, or EC, in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients age five years and over in the 31 member states of the European Economic Area, or EEA. This marketing authorization is subject to annual review and renewal by the European Medicines Agency, or EMA, following its reassessment of the risk-benefit balance of the authorization and is further conditioned on the Company’s submission of the final report, including additional safety and efficacy data, from its global, confirmatory Phase 3 clinical trial in nmDMD, which it refers to as ACT DMD, during 2015. See “Risk Factors - Risks Related to Regulatory Approval of our Product and our Product Candidates” on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk-benefit balance.

In October 2015, the Company announced results from ACT DMD, including that the trial did not meet the primary efficacy endpoint of change from baseline at week 48 in distance walked in the 6-minute walk test, or 6MWT, which we also refer to as six-minute walk distance, or 6MWD, in the overall intent-to-treat, or ITT, study population, as it showed a 15 meter benefit (p=0.213) in favor of Translarna compared to placebo, which was not statistically significant. Analyses of data from pre-specified subgroups, including patients with baseline 6MWD of 300 - 400 meters, was also conducted. A pre-specified

meta-analysis was also performed of combined data from the ACT DMD and ambulatory decline phase patients from the Company's randomized, double-blind, placebo controlled, Phase 2b clinical trial evaluating the long-term safety and efficacy of Translarna in patients with nmDMD, or the Phase 2b trial. For further discussion of ACT DMD, see "Recent Developments" in Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations." See "Risk Factors - Risks Related to the Development and Commercialization of our Product and Product Candidates" on page 40 for further detail regarding how ACT DMD results could impact our ability to commercialize Translarna.

The Company launched Translarna on a commercial basis in Germany in December 2014 and in Austria, Denmark, Norway, and Sweden in 2015. The Company expects to expand its launch activities across the EEA in the fourth quarter of 2015 and in future years, subject to successful completion of pricing and reimbursement negotiations. The market access process, including pricing and reimbursement negotiations, varies from country to country and can take over 18 months in certain circumstances. Concurrently, the Company has been making Translarna available under reimbursed early access programs in selected countries where those mechanisms exist, both within Europe and in those countries outside of Europe that will reference the marketing authorization described above.

During 2015, the Company's revenues have been and are expected to be primarily generated from sales of Translarna in counties in the EEA where pricing and reimbursement approval is obtained at acceptable levels and in other territories where permitted to distribute Translarna under early access programs, or EAPs. The Company is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of September 30, 2015, the Company had an accumulated deficit of approximately $542.1 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, convertible debt financings, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates.

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2. Summary of significant accounting policies

The Company's complete listing of significant accounting policies are described in Note 2 of the notes to the Company's audited financial statements as of December 31, 2014 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 2, 2015 (2014 Form 10-K).

Basis of Presentation

The accompanying financial information as of September 30, 2015 and for the three and nine months ended September 30, 2015 and 2014 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, 2014 and notes thereto included in the 2014 Form 10-K.

In the opinion of management, the unaudited financial information as of September 30, 2015 and for the three and nine months ended September 30, 2015 and 2014 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, 2015 are not necessarily indicative of the results to be expected for the year ended December 31, 2015 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 reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Inventories and cost of product revenue

On August 4, 2014, the Company was notified that the European Commission, or EC, granted marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. The marketing authorization allows the Company to market Translarna in the EEA. The launch in these countries is on a country by country basis. This marketing authorization is subject to annual review and renewal by the European Medicines Agency, or EMA following its reassessment of the risk-benefit balance of the authorization and is further conditioned on the Company's submission of the final report, including additional efficacy and safety data, from ACT DMD during 2015. In the third quarter of 2015, the EMA approved the annual renewal of the marketing authorization for Translarna. If we fail to satisfy renewal requirements, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require additional clinical trials. The Company does not have sufficient history or experience from which to accurately forecast product sales or demand generation. As such, the Company has not capitalized inventory and will not capitalize inventory until the completion of ACT DMD and satisfaction of the EMA conditions or until the Company can reasonably predict future product sales. The costs incurred related to the manufacturing of Translarna have been recorded as research and development expense in the consolidated statements of operations. The Company's cost of product sales includes royalties and other miscellaneous selling costs, which were not material and therefore were included as a component of research and development costs in the current period presentation. The time period over which this inventory is consumed will depend on a number of factors, including the amount of future Translarna sales, and the ability to utilize inventory prior to its expiration date.

Recently issued accounting standards

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, "Revenue from Contracts with Customers". ASU 2014-09 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 2014-09 will require that companies recognize revenue based on the value of transferred goods or services as they occur in the contract. The ASU also will 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. In July 2015, the Financial Accounting Standards Board voted to delay the effective date of this standard until the first quarter of 2018. Companies are permitted to early adopt the standard in the first quarter of 2017. Presently, the Company is assessing what effect the adoption of ASU 2014-09 will have on its financial statements and accompanying notes.

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In August 2014, the FASB issued ASU 2014-15, “Presentation of Financial Statements—Going Concern—Disclosures of Uncertainties about an entity’s Ability to Continue as a Going Concern.” ASU 2014-15 provides new guidance related to management’s responsibility to evaluate whether there is substantial doubt about an entity’s ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in U.S. auditing standards and to provide related footnote disclosures. This new guidance is effective for the annual period ending after December 15, 2016, and for annual periods and interim periods thereafter. The requirements of ASU 2014-15 are not expected to have a significant impact on its financial statements and accompanying notes.

In April 2015, the FASB issued an amendment to U.S. GAAP to simplify the balance sheet presentation of the costs for issuing debt. The changes were adopted in ASU No. 2015-03, “Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issue Costs”. Public companies will have to apply the amendments for reporting periods that begin after December 15, 2015. This amendment requires adoption by revising the balance sheets for periods prior to the effective date. The Company is currently evaluating the impact of this ASU and does not believe the adoption of this ASU will have a material impact on its financial statements and accompanying notes.

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

PTC’s net product sales have consisted solely of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. The Company applies the revenue recognition guidance in accordance with Financial Accounting Standards Board (“FASB”) Accounting Standards Codification (“ASC”) Subtopic 605-15, Revenue Recognition—Products. 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.

The Company records revenue on sales where Translarna is available either on a commercial basis or through a reimbursed early access program and typically paid for by a government authority or institution. Prior to January 1, 2015, the Company recognized revenue for commercial and reimbursed early access program sales on a cash basis once the product was shipped on behalf of the government authority or institution and payment had been received, if all other revenue recognition criteria were met. Beginning in the first quarter of 2015, the Company is recognizing revenue for Translarna as product is shipped, as the Company has established a pattern of collectability.

The Company records revenue net of estimated discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. Allowances for government rebates and discounts are established at the time of delivery. These allowances are adjusted to reflect known changes in factors that 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 the Financial Accounting Standards Board (FASB), guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, the Company evaluates if 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.

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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.

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 are reflected in the accompanying financial statements at fair value. The carrying amount of grant and collaboration 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 in this Note 3 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, 2015 and December 31, 2014:

	September 30, 2015			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 230,809	$ —	$ 230,809	$ —
Warrant liability	36	—	—	36

	December 31, 2014			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 265,493	$ —	$ 265,493	$ —
Warrant Liability	188	—	—	188

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The following is a summary of marketable securities accounted for as available-for-sale securities at September 30, 2015 and December 31, 2014:

	September 30, 2015			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Corporate debt securities	$ 198,102	$ 52	$ (987)	$ 197,167
Government obligations	33,675	17	(50)	33,642
	$ 231,777	$ 69	$ (1,037)	$ 230,809

	December 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Corporate debt securities	$ 230,379	$ 80	$ (428)	$ 230,031
Government obligations	35,501	7	(46)	35,462
	$ 265,880	$ 87	$ (474)	$ 265,493

At September 30, 2015 and December 31, 2014, 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.

Marketable securities on the balance sheet at September 30, 2015 and December 31, 2014 mature as follows:

	September 30, 2015	
	Less Than 12 Months	More Than 12 Months
Corporate debt securities	$ 144,846	$ 52,321
Government obligations	19,274	14,368
Total Marketable securities	$ 164,120	$ 66,689

	December 31, 2014

	Less Than 12 Months	More Than 12 Months
Corporate debt securities	$ 157,758	$ 72,273
Government obligations	6,003	29,459
Total Marketable securities	$ 163,761	$ 101,732

Level 3 valuation

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

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuation for warrant liability for the period ended September 30, 2015:

	Level 3 assets
Beginning balance as of December 31, 2014	$ 188
Change in fair value of warrant liability	(152)
Ending balance as of September 30, 2015	$ 36

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, 2015 include (i) volatility (62%—70%), (ii) risk free interest rate (0.49%—1.15%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($26.70), and (v) expected life (1.71—3.98 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2014 include (i) volatility (68%-70%), (ii) risk free interest rate (0.89%—1.65%), (iii) strike

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price ($128.00—$2,520.00), (iv) fair value of common stock ($51.77), and (v) expected life (2.50—4.70 years). See Note 6 for a description of the warrants issued in connection with the convertible notes.

4. Other comprehensive 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, 2015:

	Unrealized Gains/(Losses) On Marketable Securities	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at June 30, 2015	$ (486)	$ (9)	$ (495)
Other comprehensive loss before reclassifications	(483)	(149)	(632)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive loss	(483)	(149)	(632)
Balance at September 30, 2015	$ (969)	$ (158)	$ (1,127)

	Unrealized Gains/(Losses) On Marketable Securities	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2014	$ (387)	$ (350)	$ (737)
Other comprehensive income/(loss) before reclassifications	(582)	192	(390)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income/(loss)	(582)	192	(390)
Balance at September 30, 2015	$ (969)	$ (158)	$ (1,127)

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at September 30, 2015 and December 31, 2014 consist of the following:

	September 30, 2015	December 31, 2014
Employee compensation, benefits, and related accruals	$ 7,744	$ 9,312
Consulting and contracted research	9,089	9,349
Professional fees	2,975	3,334
Accounts payable	8,715	4,128
Other	3,172	2,998
	$ 31,695	$ 29,121

6. Warrants

All of the Company's outstanding warrants were classified as liabilities as of September 30, 2015 and December 31, 2014 because they contained non-standard antidilution provisions.

The following is a summary of the Company's outstanding warrants as of September 30, 2015 and December 31, 2014:

	Warrant shares	Exercise price	Expiration
Common stock	6,250	$ 128.00	2017
Common stock	7,030	$ 128.00	2019 and 2020
Common stock	130	$ 2,520.00	2019

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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,	
	2015	2014	2015	2014
Numerator				
Net loss	$ (43,223)	$ (27,282)	$ (119,499)	$ (66,484)
Denominator				
Denominator for basic and diluted net loss per share	33,908,853	29,351,693	33,528,833	28,441,827
Net loss per share:				
Basic and diluted	$ (1.27)*	$ (0.93)*	$ (3.56)*	$ (2.34)*

*In the three and nine months ended September 30, 2015 and 2014, 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,	
	2015	2014
Stock Options	4,788,264	3,443,778
Unvested restricted stock	353,135	729,320
Total	5,141,399	4,173,098

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) (see Note 9, Convertible Senior Notes). 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. For the three and nine months ended September 30, 2015, there was no dilutive effect of the Convertible Notes as the stock price did not exceed the conversion price and the Company reported a loss.

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 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.

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) 238,427 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.

From January 1, 2015 through September 30, 2015, the Company issued a total of 2,086,300 stock options to various employees. Of those, 691,100 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 our new hires' employment compensation.

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, 2014	3,432,972	$ 25.00		
Granted	2,086,300	$ 52.04		
Exercised	(654,748)	$ 13.27		
Forfeited	(74,366)	$ 42.46		
Expired	(1,894)	$ 285.48		
Outstanding at September 30, 2015	4,788,264	$ 37.64	8.58 years	$ 20,137
Vested or Expected to vest at September 30, 2015	4,497,155	$ 37.00	8.56 years	$ 19,018
Exercisable at September 30, 2015	1,132,683	$ 32.21	7.89 years	$ 10,312

The fair value of grants made in the nine months ended September 30, 2015 was contemporaneously estimated on the date of grant using the following assumptions:

	Nine months ended September 30, 2015
Risk-free interest rate	1.49% — 2.01%
Expected volatility	67%-69%
Expected term	5.50 — 6.11 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, 2015 was $32.32 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.

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

	Restricted Stock	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2015	718,400	$ 10.72
Granted	—	$ —
Vested	(361,919)	$ 10.60
Forfeited	(3,346)	$ 10.82
Unvested at September 30, 2015	353,135	$ 10.85

The Company recorded share-based compensation expense in the statement of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Research and development	$ 3,828	$ 2,363	$ 12,452	$ 6,517
General and administrative	4,226	2,258	13,678	6,088
Total	$ 8,054	$ 4,621	$ 26,130	$ 12,605

As of September 30, 2015, there was approximately $75.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, 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 2.9 years.

9. Convertible Senior 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 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

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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.

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, 2015	December 31, 2014
	(in thousands)	
Principal	$ 150,000	$ —
Less: Debt discount, net(1)	(56,802)	—
Net carrying amount	$ 93,198	$ —

(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 $93.2 million as of September 30, 2015. The Company estimates the fair value of its Convertible Notes utilizing market quotations for debt that have quoted prices in active markets. Since the Convertible Notes do not trade on a daily basis in an active market, the fair value estimates are based on market observable inputs based on borrowing rates currently available for debt with similar terms and average maturities (Level 2). As of September 30, 2015, the remaining contractual life of the Convertible Notes is approximately 6.9 years.

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

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	Three Months Ended September 30,	
	2015	2014
	(in thousands)	
Contractual interest expense	$ 584	$ —
Amortization of debt issuance costs	37	—
Amortization of debt discount	736	—
Total	$ 1,357	$ —
Effective interest rate of the liability component	11.0%	—

10. Subsequent events

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

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

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2014 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 2, 2015. 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 orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA molecule is copied from DNA through the transcription process. Our internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. 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 lead product candidate is ataluren, an investigational new drug in the United States, for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. We hold worldwide commercialization rights to ataluren for all indications in all territories. The brand name of ataluren is Translarna™.

Recent Developments — Results of ACT DMD

In October 2015, we announced results from ACT DMD, also referred to as Study 020, our Phase 3, double-blind, placebo-controlled, 48-week clinical trial to evaluate the safety and efficacy of Translarna in patients with Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD. ACT DMD involved 228 patients at 53 sites across 18 countries.

In the overall intent-to-treat, or ITT, study population, the primary endpoint of 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, showed a 15 meter benefit in favor of Translarna, which did not meet statistical significance.

However, we believe that the results of ACT DMD and the totality of clinical data across our two large, randomized, placebo-controlled trials (ACT DMD and our prior Phase 2b study, Study 007), provide substantial evidence of the effectiveness of Translarna and demonstrate a clinically meaningful benefit of Translarna for the treatment of nmDMD.

Importantly, a benefit of 47 meters was observed in the pre-specified patient population with baseline 6MWD of 300 to 400 meters, which is in line with our prior experience in the Phase 2b trial and is consistent with the evolving understanding of the natural history of the 6MWT in DMD patients. Translarna also showed a benefit over placebo in the 300 to 400 meter baseline 6MWD population across key secondary and tertiary endpoints, including timed function tests

(10 meter run/walk, four stair climb, four stair descend) and the North Star Ambulatory Assessment test. In addition, a pre-specified meta-analysis of combined data from ACT DMD and Phase 2b ambulatory decline phase patients, involving a total of 291 patients, demonstrated a statistically significant benefit in favor of Translarna in the primary endpoint of 22 meters and in key secondary endpoints. The Phase 2b ambulatory decline phase patients includes the patients from our randomized, double-blind,

placebo controlled, Phase 2b clinical trial in patients with nmDMD who would have met the enrollment criteria of ACT DMD. A meta-analysis of combined data from ACT DMD and all Phase 2b patients with baseline 6MWD of 300 to 400 meters demonstrated a 45 meter benefit in the primary endpoint as well as clinically meaningful benefits across key secondary endpoints. This meta-analysis of patients with baseline 6MWD of 300 to 400 meters was not pre-specified and is defined as post-hoc.

A summary of the safety and efficacy results from ACT DMD, including the ITT and pre-specified populations as well as the pre-specified meta-analysis of our combined ACT DMD and Phase 2b ambulatory decline phase patients is outlined below.

Safety. The results of ACT DMD confirmed the favorable safety profile of Translarna seen in the Phase 2b trial. The most common adverse events were headache, nausea and vomiting. Grade 3 adverse events were infrequent and balanced between the arms. There were no treatment-related serious adverse events. Two patients discontinued due to adverse events, including one in the Translarna arm (constipation) and one in the placebo arm (loss of ambulation).

Intent to Treat (ITT) Population. The primary efficacy endpoint in ACT DMD was change in 6MWD from baseline to week 48. In the ITT population, a 15 meter benefit (p=0.213) was observed in the primary endpoint which did not meet statistical significance.

Secondary endpoints in the trial included the proportion of patients with at least 10% worsening in 6MWD at week 48 of the trial compared to baseline, or 10% 6MWD worsening, and change in timed function tests of time to run/walk 10 meters, climb four stairs and descend four stairs. The hazard ratio for Translarna versus placebo was 0.75 (p=0.160) for 10% 6MWD worsening. Benefits trended in favor of Translarna over placebo in the timed function tests in the ITT population, including observed results in time to run/walk 10 meters (1.2 seconds; p=0.117), time to climb four stairs (1.8 seconds; p=0.058), and time to descend four stairs (1.8 seconds; p=0.012).

Additional endpoints included the North Star Ambulatory Assessment test, or NSAA test, a functional scale designed for boys affected by DMD, and the Pediatric Outcomes Data Collection Instrument, or PODCI, a validated tool for measuring quality of life in pediatric patients with orthopedic conditions. These additional endpoints favored Translarna in the ITT population but did not meet statistical significance.

Pre-Specified Analyses. The statistical analysis plan submitted to the FDA for ACT DMD set forth pre-specified analyses of efficacy to be conducted, including subgroups of patients with baseline 6MWD less than 350 meters and patients with baseline 6MWD of greater than or equal to 300 and less than 400 meters, which we refer to as our key subgroups.

The pre-specification of our key subgroups was scientifically justified based upon knowledge of the biology of the disease and the evolving understanding of the natural history of the six minute walk test in DMD patients. We considered the pre-specified less than 350 meter baseline 6MWD population as a key subgroup based on the knowledge that 350 meters represents a transition point for patients towards a more rapid decline in walking ability as supported by analysis from our Phase 2b study. Furthermore, we considered the pre-specified 300 to 400 meter baseline 6MWD population as a key subgroup based on an increasing understanding of the sensitivity limitations of the six minute walk test as an endpoint in 48-week studies. Natural history data suggests that the 6MWT may not be the optimal tool to demonstrate efficacy in patients with either a baseline 6MWD of less than 300 meters, as these patients have significant muscle loss as monitored by magnetic resonance spectroscopy and are at high risk for losing ambulation regardless of treatment, or in high walking patients, such as those with a baseline 6MWD at or greater than 400 meters, as these patients are likely to remain stable over a 48 week testing period.

By defining these key subgroups, we thereby also defined corresponding complement subgroups of patients with baseline 6MWD greater than or equal to 350 meters, greater than or equal to 400 meters, and less than 300 meters. We also pre-specified a meta-analysis of the combined results from ACT DMD and the Phase 2b ambulatory decline phase patients.

Pre-specified sub-group analysis. We saw strong evidence of clinical benefit in the pre-specified subgroup of patients with baseline 6MWD between 300 and 400 meters. Specifically, we observed a benefit in Translarna-treated patients of 47 meters (nominal p=0.007) in the 6MWT in this subgroup. This benefit was consistent with an observed benefit of 49 meters (nominal p=0.026) in our Phase 2b clinical trial in the 300 to 400 meters baseline 6MWD population. We also saw clinically meaningful benefit for Translarna over placebo in each of the timed function tests, including observed results in time to run/walk 10 meters (2.1 seconds; nominal p=0.066), time to climb four stairs (3.6 seconds; nominal p=0.003), and time to descend four stairs (4.3 seconds; nominal p<0.001). The hazard ratio for Translarna versus placebo was 0.79 (nominal p=0.418) for 10% 6MWD worsening. In addition, a benefit of 4.5 points over placebo (nominal p =0.041) was observed in the NSAA test, which we believe is clinically meaningful. We believe that the benefits observed in this key pre-specified subgroup support the of the use of the 6MWT in the patients with a walking ability in the 300 to 400 meters range and the understanding that the reliability of the 6MWT over a 48 week period was limited at both the lower and upper ends of our 6MWD enrollment range.

In the pre-specified subgroup of patients with baseline 6MWD less than 350 meters, we observed a benefit of 24 meters (nominal p=0.210) in favor of Translarna in the 6MWT. An analysis of the results from our Phase 2b clinical trial in the less than 350 meters baseline 6MWD population, defined post-hoc, demonstrated a 68 meter benefit in the 6MWT (nominal p=0.006). In the timed function tests for the subgroup of ACT DMD patients with baseline 6MWD less than 350 meters, we observed benefits for Translarna over placebo in time to run/walk 10 meters (2.3 seconds; nominal p=0.033), time to climb four stairs (4.2 seconds; nominal p=0.019) and time to descend four stairs (4.0 seconds; nominal p=0.007).

As described above, we believe the 6MWT lacks sensitivity to detect a clinical effect in patients with baseline less than 300 meters in a 48-week trial. However, the timed function tests trended in favor of patients treated with Translarna with a baseline 6MWD below 300 meters, including observed benefit over placebo in time to run/walk 10 meters (2.5 seconds; nominal p=0.066), time to climb four stairs (2.4 seconds; nominal p=0.790), and time to descend four stairs (2.1 seconds; nominal p=0.595). We believe the positive trends in this population reflect that short muscle burst activity tests may be a better clinical measure for patients that are at a more advanced stage of disease progression. Consistent with the natural history of ambulatory DMD patients with 6MWD greater than 400 meters, which indicates stability in walking ability over a 48 week period, we observed no meaningful difference in 6MWT between patient groups. Similarly, we observed no meaningful difference in 6MWT between patient groups with baseline 6MWD greater than 350 meters.

Pre-specified meta-analysis. The meta-analysis of efficacy results from the ACT DMD ITT population and Phase 2b ambulatory decline phase subgroup demonstrated a statistically significant 22 meter improvement in 6MWD (p = 0.015) favoring Translarna. Additionally, the meta-analysis showed statistically significant benefit for Translarna over placebo across each timed function test including time to run/walk 10 meters (1.4 seconds; p=0.025), time to climb four stairs (1.6 seconds; p =0.018) and time to descend four stairs (2.0 seconds; p=0.004). The hazard ratio for Translarna versus placebo was 0.66 (p=0.023) for 10% 6MWD worsening. We believe that we are able to demonstrate a statistically significant efficacy outcome in the 6MWD in the meta-analysis, despite the significant variability in baseline 6MWD among patients in both ACT DMD and the Phase 2b ambulatory decline phase, due to the substantially larger patient population available in the pooled analysis.

Retrospective Analysis. We also looked back at the observed results in the meta-analysis for all patients with a baseline 300 to 400 meter 6MWD from ACT DMD and the Phase 2b trial. The meta-analysis of this data demonstrated a 45 meter benefit (nominal p<0.001) in the 6MWT as well as clinically meaningful benefits across each secondary endpoint timed function test, including benefit over placebo in time to run/walk 10 meters (2.2 seconds; nominal p=0.008), time to climb four stairs (3.4 seconds; nominal p<0.001) and time to descend four stairs (4.3 seconds; nominal p<0.001). This meta-analysis of patients with baseline 6MWD of 300 to 400 meters was not pre-specified and is defined post-hoc.

Statistical Considerations. The pre-specified meta-analysis results, which favored Translarna in the 6MWT and each of the timed function tests, are considered statistically significant. In the pre-specified subgroups of ACT DMD patients with a baseline 6MWD less than 350 meters and 300 to 400 meters, the p-values for the 6MWT and each of the timed function tests are considered nominal. For information with respect to the use of nominal p-values and post-hoc analyses, see the risk factor set forth on page 44.

Participation Criteria and Stratification. Certain key inclusion criteria were specified in the ACT DMD trial protocol for enrollment: the patient had to be 7 through 16 years of age; at the screening visit the patient had to be able to walk no more than 80% of predicted 6MWD compared to healthy boys matched for age and height, but have the ability to walk at least 150 meters during the 6MWT; and the patient must have used systemic corticosteroids for a minimum of six months prior to start of treatment. The ACT DMD trial protocol provided for the exclusion of patients from the trial if, among other things, they recently used systemic aminoglycoside antibiotics, recently initiated or changed corticosteroid therapy or previously received Translarna treatment. Patients enrolled in ACT DMD underwent 48 weeks of blinded treatment prior to the final analysis and the randomization was stratified based on age (<9 years versus ≥9), baseline 6MWD (<350 versus ≥350 meters), and duration of prior use of corticosteroids (<12 months versus ≥12 months).

Overview and Corporate Update

Translarna™ for nonsense mutation Duchenne muscular dystrophy

Translarna™ (ataluren) received marketing authorization from the European Commission, or EC, in August 2014 for nmDMD in ambulatory patients age five years and over, in the 31 member states of the European Economic Area, or EEA. Our authorization in the EEA is subject to annual review and renewal by the European Medicines Agency, or EMA, following its reassessment of the risk benefit balance of the authorization, which we refer to as the annual EMA reassessment. This marketing authorization is further conditioned on our submission of the final report from our ACT DMD trial, including additional efficacy and safety data, during 2015. Upon fulfilment of this specific obligation to submit the ACT DMD report, in the event that the EMA determines that the positive risk benefit balance for Translarna is confirmed, the EMA may recommend the granting of a marketing authorization that is no longer subject to any further specific obligations, but will generally continue to be subject to periodic renewal requirements in line with applicable regulations in the EEA. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance.

We launched Translarna on a commercial basis in Germany in December 2014 and in Austria, Denmark, Norway and Sweden in 2015. We expect to expand our launch activities across the EEA through the fourth quarter of 2015 and in future years, subject to successful completion of pricing and reimbursement negotiations. The market access process, including pricing and reimbursement negotiations, varies from country to country and can take over 18 months in certain circumstances.

Concurrently, we have been making available Translarna for the treatment of nmDMD through reimbursed early access programs, or EAP programs, in selected countries where funded named patient or cohort programs exist, both within the EEA and in those countries outside of the EEA that will reference the marketing authorization in the EEA. Translarna has been made available under EAP programs in Brazil, Colombia, France, Greece, Italy, Israel, Spain, and Turkey.

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During the fourth quarter of 2014 we initiated a rolling new drug application, or NDA, with the U.S. Food and Drug Administration, or FDA, for Translarna for the treatment of nmDMD. We believe this process gives the FDA an opportunity to conduct a meaningful review of most of the segments of our NDA, ahead of reviewing our ACT DMD data. We expect that the submission of our ACT DMD data will complete our rolling NDA and allow for FDA review.

If granted expedited review and approval by the FDA, among other factors, we believe we have the potential to begin commercialization of Translarna in the United States in the first half of 2016. In preparation for a potential U.S. launch, subject to positive ACT DMD data and receipt of FDA approval, we have begun building out our commercial team and infrastructure in the United States.

We ultimately intend to market Translarna in all markets in the EEA where market access is possible. We currently expect Translarna to be priced at levels consistent with the pricing for other therapies for the treatment of rare disorders where high unmet medical need exists.

Translarna™ for nonsense mutation cystic fibrosis

Screening has now closed for our global, confirmatory Phase 3 clinical trial of Translarna for the treatment of cystic fibrosis caused by nonsense mutations, or nmCF. We refer to this trial as ACT CF. We anticipate ACT CF enrollment will be completed by the end of 2015 with top-line data about a year later.

During the third quarter of 2015, we submitted a variation to our marketing authorization of Translarna in the EEA, described above, to request approval of Translarna for the treatment of nmCF. Approval of the marketing authorization variation will depend on the EMA's assessment of the relative benefits and risks of approval. We may not be able to demonstrate the required relative risk-benefit profile and there is substantial risk that the EMA will not grant us a

variation approving Translarna for the treatment of nmCF. If approved, we expect that the EMA will require us, as a post-approval measure, to provide comprehensive clinical data from ACT CF to the EMA. In addition, unless and until we satisfy the conditions of our primary marketing authorization in the EEA for Translarna, such authorization will continue to be subject to annual review and renewal by the EMA. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance.

Our variation submission to the EMA for Translarna for the treatment of nmCF included recent retrospective analyses of the data from our previous Phase 3 trial completed in 2011. Natural history data for cystic fibrosis suggests that patients under 18 years of age experience more rapid rates of decline in pulmonary function. In addition, as previously reported, post hoc analysis of data from our previous Phase 3 trial indicates that patients not using chronic inhaled tobramycin experienced a benefit in relative change from baseline in percent-predicted FEV_1 favoring the Translarna-treated group versus placebo at week 48, with fewer pulmonary exacerbations in the Translarna-treated group. Given the natural history of the disease and the data from our previous Phase 3 trial, we undertook further analyses to compare pulmonary outcomes on the basis of age (under 18 years versus 18 years or older) in patients who did not use chronic inhaled tobramycin. In this new subgroup analysis, non-TOBI Translarna patients under 18 showed an increase in FEV_1 over baseline, a mean relative change in predicted FEV_1 of 8.4% and an absolute change in predicted FEV_1 of 5.4%. In addition, non-TOBI patients under 18 years receiving Translarna also showed a 60% reduction in exacerbation rate. For additional considerations see our risk factor with respect to retrospective analyses of clinical trial results on page 44.

Translarna™ for additional indications

Over the last six years multiple independent investigators have conducted preclinical studies in which Translarna enabled readthrough of the premature stop codons from a large set of nonsense mutations across a diverse group of experimental models exhibiting various genetic disorders. The studies evaluated Translarna's ability to read through premature stop codons in mRNA in cell-free systems, transfected cell lines, mouse models and patient cells. Based on these studies by independent investigators in addition to our own trials and studies, we expect to continue to pursue additional indications for Translarna, including nmMPS I and aniridia caused by nonsense mutation.

MPS I is an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of byproducts of chemical reactions in the body's cells. It is estimated that 60 percent to 80 percent of cases of MPS I are caused by a nonsense mutation. While enzyme replacement therapies are on the market, there remains significant unmet medical need for the development of new treatments that can target the underlying cause of the disorder.

During the first quarter of 2015, we amended the trial design for our proof-of-concept study for Translarna for the treatment of nmMPS I to include patients currently on enzyme replacement therapy. We anticipated that this change in protocol might cause delays in patient enrollment, but expected that the larger addressable patient population would reduce the time to enroll the overall study.

However, this protocol revision resulted in delays to site initiation and patient accrual and we no longer expect data from this trial to be available by the end of 2015.

Aniridia is a genetic disorder due to mutations in the PAX6 gene associated with loss of eyesight and other symptoms. We estimate that approximately one-third of all aniridia cases are due to a nonsense mutation. In a prior study conducted by an independent investigator, Translarna-treated mice with nonsense mutation aniridia showed a significant increase in the PAX6 protein in a nonsense mutation PAX6 gene, but not in a PAX6 gene harboring a splice-site mutation. The investigators in this study found that Translarna not only inhibited disease progression, but also reversed corneal, lens and retinal malformation defects and restored electrical responses of the retina.

During the fourth quarter of 2015, we submitted an investigational new drug, or IND, application to the FDA for the initiation of clinical assessment of Translarna in nonsense mutation aniridia.

Spinal muscular atrophy program

RG7800, an investigational oral therapy for spinal muscular atrophy, or SMA, is our product candidate under our joint development SMA program 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.

Results from the Phase 1 study of RG7800, which was completed in the first half of 2014, were presented at the emerging science session at the 2015 annual meeting of the American Academy of Neurology. The Phase 1 study was a single ascending dose, placebo-controlled clinical study in 48 healthy volunteers. Results from the Phase 1 study indicated that RG7800 showed a favorable safety profile and was well tolerated at all dose levels studied. In addition, proof of mechanism was demonstrated by a dose-dependent effect on Survival Motor Neuron 2 (SMN2) mRNA splicing towards the production of full length SMN2 mRNA.

MOONFISH, a Phase 2, multiple-dose clinical study investigating the safety and tolerability of RG7800 was initiated in November 2014. Secondary outcome measures include pharmacokinetics and pharmacodynamics as well as exploratory efficacy endpoints. Dosing of the first cohort of the Phase 2 MOONFISH study was completed in the second quarter of 2015.

Results from the first cohort of patients enrolled in the MOONFISH study were highlighted in an oral session at the International Congress of the World Muscle Society in October 2015. Results from the first cohort demonstrated that treatment with RG7800 shifts SMN2 splicing toward the production of full length SMN mRNA and generated up to two-fold increases in SMN protein in patients with SMA. RG7800 was well tolerated during the first cohort period of 12 weeks at a dose of 10 mg once daily.

During the second quarter of 2015, the collaboration partners decided to suspend dosing of patients in MOONFISH to evaluate data from a 39-week non-clinical safety and toxicology study of RG7800 in cynomolgus monkeys which showed an eye finding at exposures above those explored in SMA patients and healthy volunteers.

Pre-clinical investigations regarding our lead compound, RG7800, are ongoing. Concurrent with the advancement of our lead compound, a robust research effort regarding SMN2 splicing has continued to advance through IND-enabling studies. Additional data are expected in the coming months which will be utilized to determine the best clinical development path forward for the SMA program. We expect that clinical development will resume in early 2016.

Cancer stem cell program

A Phase 1 first-in-human, dose-escalation safety and pharmacokinetic open-label clinical study for our product candidate, PTC596, in advanced cancer patients with solid tumors initiated in April 2015 and is ongoing. PTC596 is a first-in-class, orally bioavailable and potent small molecule that targets tumor stem cell populations by reducing the function, activity and amount of a protein called BMI1. Elevated levels of BMI1 are associated with more aggressive tumors and a poor prognosis in a wide variety of cancers, including glioblastoma.

Antibacterial program

Based on pre-clinical safety signals, we are no longer advancing PTC672. We are considering the status of our antibacterial program and the timing of potential advancement of a new compound for the treatment of life-threatening infections caused by multi-drug resistant Gram-negative bacteria.

Funding

During 2015, our revenues have been and are expected to continue to be primarily generated from sales of Translarna in counties in

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the EEA where we are able to obtain pricing and reimbursement approval at acceptable levels and in other territories where we are permitted to distribute Translarna under our EAPs.

We have financed our operations primarily through our private offering of 3.00% convertible senior notes due 2022 in August 2015 (which we refer to as the Convertible Notes), 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.

As of September 30, 2015, we had an accumulated deficit of $542.1 million. We had a net loss of $119.5 million for the nine months ended September 30, 2015 and a net loss of $66.5 million for the nine months ended September 30, 2014.

We anticipate that our expenses will increase substantially in connection with the expansion of our commercial infrastructure as we continue to establish an international presence, particularly throughout Europe and in the U.S., and commercialize Translarna for the treatment of nmDMD, including significant sales and marketing, legal and regulatory, and distribution and manufacturing expenses. In addition, we expect to continue to incur significant costs in connection with our ongoing confirmatory Phase 3 ACT CF clinical trial of Translarna as well as our Phase 2 proof-of-concept studies for nmMPS I and nonsense mutation aniridia. We also expect to incur ongoing research and development expenses for our other product candidates, including our antibacterial program and the ongoing Phase 1 clinical study under our cancer stem cell program. In addition, we may incur substantial costs in connection with our ongoing rolling NDA submission with the FDA for Translarna for the treatment of nmDMD, our expected submission of the final report on ACT DMD to the EMA, and our submitted marketing authorization variation with the EMA, which seeks to include Translarna for the treatment of nmCF. We have begun seeking and intend to continue to seek marketing approval for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing approval for Translarna for other indications, and these efforts may significantly impact the timing and extent of our commercialization expenses.

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.

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. We will need to generate significant revenues to achieve and sustain profitability, and we may never do so.

Financial operations overview

To date, our net product sales have consisted solely of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. Our process for recognizing revenue is described below under “Critical accounting policies and significant judgments and estimates—Revenue recognition”.

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

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

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

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

Grant revenue. From time to time, we receive grant funding from various institutions and governmental bodies. The grants are

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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, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

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

We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we continue our confirmatory Phase 3 ACT CF clinical trial of Translarna, our Phase 2 proof-of-concept study of Translarna in nmMPS I and nonsense mutation aniridia, and our Phase 1 clinical study under our cancer stem cell program. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs and product candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs, for the three and nine months ended September 30, 2015 and September 30, 2014.

	Three months September 30,	
	2015	2014
	(in thousands)	
Translarna (nmDMD, nmCF and nmMPS I)	$ 19,600	$ 11,211
Antibacterial	2,527	1,636
Cancer stem cell	1,736	588
Spinal muscular atrophy	269	518
Next generation nonsense readthrough	1,857	1,705
Other research and preclinical	4,651	3,107
Total research and development	$ 30,640	$ 18,765

	Nine months September 30,	
	2015	2014
	(in thousands)	
Translarna (nmDMD, nmCF and nmMPS I)	$ 54,524	$ 29,831
Antibacterial	7,542	5,786
Cancer stem cell	5,413	1,773
Spinal muscular atrophy	906	2,058
Next generation nonsense readthrough	5,958	4,770
Other research and preclinical	12,425	8,749
Total research and development	$ 86,768	$ 52,967

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

- the scope, rate of progress and expense of our clinical trials and other research and development activities;
- the potential benefits of our products and product candidate over other therapies;

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- our ability to market, commercialize and achieve market acceptance for any of our product candidates that we are developing or may develop in the future;
- clinical trial results;

- the terms and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of Translarna or any other product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of 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, and accounting services.

We expect that selling, general and administrative expenses will increase in future periods as a result of our continued efforts to establish an expanded international presence in Europe and other territories 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 income consists of interest income earned on investments and interest expense from the Convertible Notes outstanding.

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 solely of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. We began recognizing revenue for payments received under the reimbursed EAPs for Translarna in nmDMD patients in selected countries in the third quarter of 2014. We have now established a pattern of collectability and, beginning in January 2015, 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 ("FASB") Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition—Products.

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We have recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program and typically paid for by a government authority or institution. 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, the Company is recognizing revenue for Translarna as product is shipped, as the Company has established a pattern of collectability.

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

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

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

Inventories and Cost of Product Revenues

In 2014, we were notified that the European Commission, or EC, granted marketing authorization for Translarna for the treatment of nmDMD, in ambulatory patients aged five years and older. This marketing authorization is subject to annual review and renewal by the EMA following its reassessment of the risk-benefit balance of the authorization, which we refer to as the annual EMA reassessment, and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD in 2015 and our ability to implement measures, including pharmacovigilance plans, that are detailed in the risk management plan for Translarna that was submitted to EMA. In the third quarter of 2015, the EMA approved the annual renewal of the marketing authorization for Translarna for the treatment of nmDMD. We plan to seek to renew the approval on an annual basis until our obligations have been fulfilled and the approval is converted from a conditional approval into a full approval. If we fail to satisfy such requirements, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for

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Translarna or require additional clinical trials. The conditional marketing authorization allows us to market Translarna in the 31 member states of European Economic Area. Our launch in these countries is on a country by country basis.

We do not have sufficient history or experience from which to accurately forecast product sales or demand generation. As such, we have not capitalized inventory and will not capitalize inventory until full approval has been obtained or until we can reasonably predict future product sales and demand generation. The Company's cost of product sales consists principally of royalties that are less than $0.2 million and included as a component of research and development costs in the current year presentation.

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.

During the nine months ended September 30, 2015, we issued a total of 2,086,300 stock options to various employees. Of those, 691,100 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, 2015 was contemporaneously estimated on the date of grant using the following assumptions:

	Nine months ended September 30,	
	2015	2014
Risk-free interest rate	1.49%—2.01%	0.11%—2.04%
Expected volatility	67%—69%	79%-91%
Expected term	5.50—6.11 years	5.50—6.25 years

We assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the nine months ended September 30, 2015 was $32.32 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

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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.

The following table summarizes information on our restricted stock:

	Restricted Stock	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2015	718,400	$ 10.72
Granted	—	—
Vested	(361,919)	$ 10.60
Forfeited	(3,346)	$ 10.82
Unvested at September 30, 2015	353,135	$ 10.85

We recorded share-based compensation expense in the statement of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Research and development	$ 3,828	$ 2,363	$ 12,452	$ 6,517
General and administrative	4,226	2,258	13,678	6,088
Total	$ 8,054	$ 4,621	$ 26,130	$ 12,605

As of September 30, 2015, there was approximately $75.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, 2013 Long Term Incentive Plan and equity award plans 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 2.9 years.

Convertible Notes Offering

In August 2015, we issued, at par value, $150.0 million aggregate principal amount of 3.0% convertible senior notes due 2022. 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 us from the offering were $145.4 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by us.

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: (1) during any calendar quarter commencing on or after September 30, 2015 (and only during such calendar quarter), if the last reported sale price of our 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; (2) 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 our common stock and the conversion rate on each such trading day; (3) during any period after we have issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or (4) 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, we 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.

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The conversion rate for the Convertible Notes was initially, and remains, 17.7487 shares of our 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 our common stock.

We may not redeem the Convertible Notes prior to August 20, 2018. We 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 we provide 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 we are not required to redeem or retire the Convertible Notes periodically.

If we undergo a “fundamental change” (as defined in the Indenture governing the Convertible Notes Indenture), subject to certain conditions, holders of the Convertible Notes may require us 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 our 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 us, or the holders of at least 25% in principal amount of the outstanding Convertible Notes by notice to us 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 us 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.

Results of operations

Three months ended September 30, 2015 compared to three months ended September 30, 2014

The following table summarizes revenues and selected expense and other income data for the three months ended September 30, 2015 and 2014.

(in thousands)	Three months ended September 30, 2015	2014	Change 2015 vs. 2014
Net product revenue	$ 9,772	$ 81	$ 9,691
Collaboration and grant revenue	4	1,613	(1,609)
Research and development expense	30,640	18,765	11,875
Selling, general and administrative expense	21,368	10,530	10,838
Interest (expense)/income, net	(852)	354	(1,206)

Net product revenues. Net product revenues were $9.8 million for the three months ended September 30, 2015. We have recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program and typically paid for by a government authority or institution. As of January 1, 2015, we recognized revenue for Translarna as product is shipped, given we have established a pattern of collectability.

Collaboration and grant revenues. Collaboration and grant revenues decreased by $1.6 million, or 100%, from $1.6 million for the three months ended September 30, 2014. The decrease primarily relates to lower collaboration revenue from the research component of the collaboration with Roche and the SMA Foundation.

Research and development expense. Research and development expense was $30.6 million for the three months ended September 30, 2015, an increase of $11.9 million, or 63%, from $18.8 million for the three months ended September 30, 2014. The increase was primarily due to expansion of our clinical development activities including late-stage studies in both nmDMD and nmCF as well as from an increase in non-cash, share-based compensation expense of approximately $1.5 million.

Selling, general and administrative expense. Selling, general and administrative expense was $21.4 million for the three months ended September 30, 2015, an increase of $10.8 million, or 103%, from $10.5 million for the three months ended September 30, 2014. The

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increase resulted primarily from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions and from an increase in non-cash, share-based compensation expense of approximately $2.0 million.

Interest expense/income, net. Interest expense, net was $0.9 million for the three months ended September 30, 2015, a decrease of $1.2 million, or 341%, from $0.4 million interest income, net for the three months ended September 30, 2014. The decrease was due to interest expense recorded from the Convertible Notes offering in August 2015.

Income tax expense. Income tax expense was $0.1 million for the three months ended September 30, 2015 and $0 for the three months ended September 30, 2014. The increase in income tax expense primarily relates to certain foreign income taxes. 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, 2015 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 impact 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 forecasted permanent book-to-tax differences, and changes resulting from the impact of tax law changes.

Nine months ended September 30, 2015 compared to nine months ended September 30, 2014

The following table summarizes revenues and selected expense and other income data for the nine months ended September 30, 2015 and 2014.

(in thousands)	Nine months ended September 30, 2015	2014	Change 2015 vs. 2014
Net product revenue	$ 21,002	$ 81	$ 20,921
Collaboration and grant revenue	3,030	12,506	(9,476)
Research and development expense	86,768	52,967	33,801
Selling, general and administrative expense	56,193	26,803	29,390
Interest (expense)/income, net	170	774	(604)

Net product revenues. Net product revenues were $21.0 million for the nine months ended September 30, 2015, which includes $1.4 million of revenue attributable to product sales in 2014 which were deferred in 2014 when we recognized revenue on a cash basis. We have recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program and typically paid for by a government authority or institution. As of January 1, 2015, we recognized revenue for Translarna as product is shipped, given we have established a pattern of collectability.

Collaboration and grant revenues. Collaboration and grant revenues were $3.0 million for the nine months ended September 30, 2015, a decrease of $9.5 million, or 76%, from $12.5 million for the nine months ended September 30, 2014. The decrease was primarily due to recognition of a $7.5 million milestone in our spinal muscular atrophy collaboration with Roche in the first quarter of 2014, which was non-recurring, and lower collaboration revenue for the comparable period.

Research and development expense. Research and development expense was $86.8 million for the nine months ended September 30, 2015, an increase of $33.8 million, or 64%, from $53.0 million for the nine months ended September 30, 2014. The increase resulted primarily from an increase in clinical trial related expenses associated with our ongoing clinical trials, supply chain activities in support of the launch of Translarna and increased expenses in connection with our expanding clinical-stage pipeline and an increase in non-cash, share-based compensation expense of approximately $5.9 million.

Selling, general and administrative expense. Selling, general and administrative expense was $56.2 million for the nine months ended September 30, 2015, an increase of $29.4 million, or 110%, from $26.8 million for the nine months ended September 30, 2014. The increase resulted primarily from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions and from an increase in non-cash, share-based compensation expense of approximately $7.6 million.

Interest (expense)/income, net. Interest income, net was $0.2 million for the nine months ended September 30, 2015, a decrease of $0.6 million, or 78%, from $0.8 million for the nine months ended September 30, 2014. The decrease was due interest expense recorded from the Convertible Notes offering in August 2015.

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Income tax expense. Income tax expense was $0.2 million for the nine months ended September 30, 2015 and $0 for the nine months ended September 30, 2014. The increase in income tax expense primarily relates to certain foreign income taxes. 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, 2015 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 impact 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 forecasted 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 while also devoting a substantial portion of our efforts on research and development programs related to Translarna and our other product candidates. During 2015, we expect that our revenues will be primarily generated from sales of Translarna in counties in the EEA where we are able to obtain pricing and reimbursement approval at acceptable levels and in other territories where we are permitted to distribute Translarna under our early access programs, or EAP.

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. We expect to continue to incur significant expenses and operating losses for at least the next several years. The net losses we incur may fluctuate significantly from quarter to quarter.

In February 2014, we closed a public offering of 5,163,265 shares of common stock at a public offering price of $24.50 per share, including 673,469 shares pursuant to the exercise by the underwriters of an overallotment option. We received net proceeds from the public offering of approximately $118.4 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

In October 2014, we closed a public offering of 3,450,000 shares of common stock at a public offering price of $36.25 per share, including 450,000 shares pursuant to the exercise by the underwriters of their option to purchase additional shares. We received net proceeds from the public offering of approximately $117.6 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

In August 2015, we closed a private offering of $150 million in aggregate principal amount of 3.00% convertible senior notes due 2022, or the Convertible Notes, 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 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 PTC.

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Cash flows

As of September 30, 2015, we had cash, cash equivalents and marketable securities of $371.5 million.

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

	Nine months ended Sepember 30,	
(in thousands)	**2015**	**2014**
Cash provided by (used in):		
Operating activities	$ (94,005)	$ (49,281)
Investing activities	31,019	(31,983)
Financing activities	154,039	118,723

Net cash used in operating activities was $94.0 million for the nine months ended September 30, 2015 and $49.3 million for the nine months ended September 30, 2014. The change in net cash used in operating activities primarily related to supporting clinical development and commercial activities.

Net cash provided by investing activities was $31.0 million for the nine months ended September 30, 2015 and net cash used in investing activities was $32.0 million for the nine months ended September 30, 2014. Cash provided by investing activities was related to the sale & redemption of marketable securities for the nine months ended September 30, 2015. Cash used in investing activities was related to net purchases of investments for the nine months ended September 30, 2014.

Net cash provided by financing activities was $154.0 million for the nine months ended September 30, 2015 and $118.7 million for the nine months ended September 30, 2014. Net cash provided by financing activities was primarily attributable to approximately $145.4 million in net proceeds from the issuance of convertible notes in August 2015 for the nine months ended September 30, 2015. Net cash provided by financing activities was primarily attributable to approximately $118.4 million in net proceeds from the February 2014 public offering for the nine months ended September 30, 2014.

Funding requirements

We anticipate that our expenses will increase substantially in connection with the expansion of our commercial infrastructure as we continue to establish an international presence, particularly throughout Europe and in the United States, and commercialize Translarna for the treatment of nmDMD, including significant sales and marketing, legal and regulatory, and distribution and manufacturing expenses. In addition, we expect to continue to incur significant costs in connection with our ongoing confirmatory Phase 3 ACT CF clinical trial of Translarna as well as our Phase 2 proof-of-concept studies for nmMPS I and nonsense mutation aniridia. We also expect to incur ongoing research and development expenses for our other product candidates, including our antibacterial program and the ongoing Phase 1 clinical study under our cancer stem cell program. In addition, we may incur substantial costs in connection with our ongoing rolling NDA submission with the FDA for Translarna for the treatment of nmDMD , our expected submission of the final report on ACT DMD to the EMA, and our submitted marketing authorization variation with the EMA, which seeks to include Translarna for the treatment of nmCF. We have begun seeking and intend to continue to seek marketing approval for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing approval for Translarna for other indications, and these efforts may significantly impact the timing and extent of our commercialization expenses.

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

- 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;
- 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 Convertible Notes 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:

- the costs, timing and outcome of regulatory review of Translarna and our other product candidates, including those related to our rolling NDA submission with the FDA for Translarna for the treatment of nmDMD, our marketing authorization for Translarna for the treatment of nmDMD in the EEA, and our request for marketing authorization variation with the EMA for Translarna for the treatment of nmCF;
- whether the FDA, the EMA and regulators in other territories agree with our interpretation of the results of ACT DMD;
- the progress and results of our confirmatory Phase 3 ACT CF clinical trial of Translarna as well as our Phase 2 proof of concept studies for nmMPS I and nonsense mutation aniridia and our ongoing Phase 1 clinical study under our cancer stem cell program;
- the scope, costs and timing of the expansion of our commercial infrastructure, including in connection with the growth of our international presence in Europe and in other territories;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, in the European Economic Area for nmDMD and any of our other product candidates that may receive marketing approval or any additional indications or territories in which we receive authorization to market Translarna;
- 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;
- the number and development requirements of other product candidates that we pursue;
- revenue received from commercial sales of Translarna or any of our other product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products and technologies; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

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.

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.

Contractual obligations

During the period ending September 30, 2015, 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, 2014.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ending September 30, 2015, 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, 2014.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2015. 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, 2015, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended September 30, 2015 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

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.

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Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to incur significant expenses in connection with the continued expansion of our global operations and execution of our commercial strategy for Translarna™ (ataluren) in the European Economic Area and other territories, our efforts to obtain broader and additional regulatory approvals for Translarna, and the development of our product pipeline. We expect to continue to incur operating losses for at least the next several years and may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. As of September 30, 2015, we had an accumulated deficit of $542.1 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 candidates. We expect to continue to incur significant expenses and operating losses for at least the next several years. The net losses we incur may fluctuate significantly from quarter to quarter.

We are a growing commercial-stage biopharmaceutical company, but prior to 2014 we devoted substantially all of our efforts on research and development, including clinical trials. In August 2014, the European Commission granted marketing authorization for Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The marketing authorization is subject to annual review and renewal by the EMA following its reassessment of the risk-benefit balance of the authorization, which we refer to as the annual EMA reassessment, and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD during 2015. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance.

We announced the initial results of ACT DMD in October 2015. While the primary efficacy endpoint in the intent to treat population, or ITT, did not achieve statistical significance, we believe 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. There is substantial risk that the FDA, the EMA and other regulators 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 from the sales of Translarna for the treatment of nmDMD. See "Risk Factors - Risks Related to the Development and Commercialization of our Product and Product Candidates" on page 40 for further detail regarding how the ACT DMD results could impact our ability to commercialize Translarna.

The marketing authorization described above allows us to market Translarna in the 31 member states of the European Economic Area, or EEA. We commenced our commercial launch of Translarna in Germany in December 2014 and we expect to commercially launch in other key countries in the EEA through the fourth quarter of 2015 and in future years, subject to completion of pricing and reimbursement negotiations. Concurrently, in preparation for a potential U.S. launch in the first half of 2016, we have begun building out our commercial team and infrastructure in the United States. We anticipate that our

expenses will increase substantially in connection with the expansion of our global infrastructure as we continue to establish an international presence and commercialize Translarna for the treatment of nmDMD, including significant sales and marketing, legal and regulatory, and distribution and manufacturing expenses.

In addition, we expect to continue to incur significant costs in connection with our confirmatory Phase 3 ACT CF clinical trial of Translarna as well as our Phase 2 proof-of-concept studies for nmMPS I and aniridia. We also expect to incur ongoing research and development expenses for Translarna in additional indications as well as for our other product candidates, including our antibacterial program and the ongoing Phase 1 clinical study under our cancer stem cell program. In addition, we may incur substantial costs in connection with our rolling NDA submission with the FDA for Translarna for the treatment of nmDMD, our expected submission of the final report on ACT DMD to the EMA, and our submitted marketing authorization variation with the EMA, which seeks to include Translarna for the treatment of nmCF. We have begun seeking and intend to continue to seek marketing approval for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing approval 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:

- 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;
- maintain, expand and protect our intellectual property portfolio; and

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- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

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 confirmatory Phase 3 ACT CF clinical trial of Translarna;
- completing our regulatory submissions in a timely manner, including our rolling NDA with the FDA and our final report with respect to ACT DMD to the EMA for Translarna for the treatment of nmDMD;
- expanding the territories in which we are approved to market Translarna for the treatment of nmDMD, in particular in the United States;
- advancing our submitted request for a marketing authorization variation with the EMA to seek inclusion of Translarna for the treatment of nmCF;
- initiating clinical studies of Translarna for the treatment of additional indications, including nmMPS I and nonsense mutation aniridia, and successfully advancing our other programs and collaborations, including our cancer stem cell, antibacterial and SMA programs;
- establishing a global commercial infrastructure, including the sales, marketing and distribution capabilities to effectively market and sell Translarna in Europe, the United States, and other parts of the world;
- implementing marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- negotiating and securing adequate pricing and reimbursement terms for Translarna on a timely basis, or at all, in the countries in which we have and may obtain regulatory approval;
- negotiating and securing adequate reimbursement from other third-party payors for Translarna;
- launching commercial sales of Translarna for the treatment of nmDMD in accordance with our estimated timeline;
- maintaining the marketing authorization of Translarna for the treatment of nmDMD in the EEA and satisfying all related conditions and ongoing requirements;
- identifying patients eligible for treatment with Translarna;
- obtaining approval to market Translarna for the treatment of other indications;
- expanding the approved product label of Translarna for the treatment of nmDMD;
- protecting our rights to our intellectual property portfolio related to Translarna; and
- contracting for the manufacture and distribution of commercial quantities of Translarna;

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.

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We expect to incur significant expenses related to the establishment of an expanded international presence and the commercialization of Translarna, including costs related to product sales and marketing, legal and regulatory, and distribution and manufacturing, which may further increase as we expand the geographic area covered by our commercial launch and in the event we receive additional approvals for the use of Translarna or any of our other product candidates. In addition, we expect our research and development expenses to increase in connection with our ongoing activities, particularly as we continue our confirmatory Phase 3 ACT CF clinical trial of Translarna, continue to pursue our Phase 2 proof-of-concept studies of Translarna in nmMPS and aniridia caused by nonsense mutation, and our Phase 1 clinical study under our cancer stem cell program. Furthermore, since the closing of our initial public offering in June 2013, we have incurred additional costs associated with operating as a public company.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, including the net proceeds from 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:

- the costs, timing and outcome of regulatory review of Translarna and our other product candidates, including those related to our rolling NDA submission with the FDA for Translarna for the treatment of nmDMD, our marketing authorization for Translarna for the treatment of nmDMD in the EEA, and our request for marketing authorization variation with the EMA for Translarna for the treatment of nmCF;
- whether the FDA, the EMA and regulators in other territories agree with our interpretation of the results of ACT DMD;
- the progress and results of our confirmatory Phase 3 ACT CF clinical trial of Translarna as well as our Phase 2 proof of concept studies for nmMPS I and nonsense mutation aniridia and our ongoing Phase 1 clinical study under our cancer stem cell program;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, in the European Economic Area for nmDMD and any of our other product candidates that may receive marketing approval or any additional indications or territories in which we receive authorization to market Translarna;
- 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;
- the number and development requirements of other product candidates that we pursue;
- revenue received from commercial sales of Translarna or any of our other product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products and technologies; and
- our ability to establish and maintain collaborations, 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 product candidates, if approved, may not achieve commercial success, including Translarna for the treatment of nmDMD.

We are continuing to engage in significant commercialization efforts for Translarna for nmDMD throughout the EEA. We commenced our commercial launch of Translarna in Germany in December 2014 and we expect to commercially launch in other key

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countries in the EEA through the fourth quarter of 2015 and in future years, subject to completion of pricing and reimbursement negotiations. In the third quarter of 2014, we began to recognize revenue for payments received under reimbursed early access programs for Translarna for nmDMD patients in selected countries. We expect that our commercial revenue generated in the next several years will be derived exclusively from sales of Translarna for the treatment of nmDMD and other indications, if any, that may receive marketing authorization and that commercial sales will generally be limited to countries in the European Economic Area and other territories in which we have obtained marketing authorization and reimbursement approval or are permitted to initiate treatment under reimbursed early access programs or pursuant to other procedures. Other commercial revenue, if any, would be derived from sales of products that we are not planning to have commercially available for several years, if at all.

Accordingly, we will need to continue to rely on additional financing 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.

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, 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.

In the third quarter of 2014 we began to recognize revenue for payments received under reimbursed early access programs for Translarna for nmDMD patients in selected countries, and we commenced our commercial launch of Translarna in Germany in December 2014. Prior to such time, our operations were limited to organizing and staffing our company, developing and securing our technology, raising capital, undertaking preclinical studies and clinical trials of our product candidates, and preparing for the commercial launch of Translarna for nmDMD in Europe. We are in the process of 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 such a transition. 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 EMA reassessment until we fulfill certain obligations, we have not proven our ability to successfully obtain marketing approvals to sell our product or product candidates. In addition, we have not yet demonstrated our ability to complete development of product candidates, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for a successful full scale product commercialization. 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.

Our ability to use our net operating losses and certain other tax attributes may be subject to annual limitations under federal and state tax law that could materially affect our ability to utilize such losses and attributes.

If a corporation undergoes an “ownership change” within the meaning of Section 382 of the Internal Revenue Code, or Section 382, 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 Section 382 that have resulted in limitations under Section 382 (and similar state provisions) on the use of our NOLs and other tax attributes.

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Future changes in ownership could result in additional ownership changes within the meaning of Section 382 that could further limit our ability to utilize our NOLs and certain other tax attributes.

Changes in our effective income tax rate 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.

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

We recently announced that ACT DMD, our Phase 3 trial for Translarna for the treatment of nmDMD, did not meet its primary efficacy endpoint and there is substantial risk that the FDA, the EMA and other 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 the initial results of ACT DMD, including that the primary efficacy endpoint in the ITT population did not achieve statistical significance. We believe 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 intend to submit our analyses of the ACT DMD data and meta-analysis of the combined ACT DMD and Phase 2b subgroup data to the FDA in connection with our rolling NDA for Translarna for the treatment of nmDMD as well as to the EMA in connection with our marketing authorization in the EEA, which is conditioned, among other things, on the submission of the final results of ACT DMD to the EMA by the end of 2015. We also intend to use these analyses to support our applications for marketing authorization for Translarna for the treatment of nmDMD in other territories.

There is substantial risk that the FDA, the EMA and other 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 ability to generate revenue from the sales of Translarna for the treatment of nmDMD. An inability to generate such revenue would have a material adverse effect impact our business, financial performance and results of operations.

For additional information, see "Risks Related to Regulatory Approval of our Product and our Product Candidates - If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to continue to commercialize Translarna for nmDMD or other indications or commercialize our other product candidates, and our ability to generate revenue will be materially impaired" on page 61.

We depend heavily on the success of our lead product, Translarna, which we are developing for nmDMD, nmCF, nmMPS I and nonsense mutation aniridia. All of our other product candidates, including those under our collaborations with Roche and the SMA Foundation, are still in early clinical or preclinical development. If we are unable to execute our commercial strategy for Translarna for the treatment of nmDMD in the European Economic Area, fail to receive regulatory approval in the United States and other territories, fail to maintain or satisfy the conditions of our marketing authorization in the European Economic Area, 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 nmCF. Additionally, we are pursuing proof of concept studies for Translarna in additional indications, nmMPS I and nonsense mutation aniridia. Our ability to generate product revenues will depend heavily on the successful development and commercialization of Translarna. In August 2014, Translarna was granted marketing authorization in the EEA for the treatment of nmDMD in ambulatory patients aged five years and older, which is subject to annual EMA reassessment and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD in 2015. Translarna is still under investigation for the treatment of nmDMD in the United States and has not been approved by the FDA.

We announced the initial results of ACT DMD in October 2015. While the primary efficacy endpoint in the ITT population did not achieve statistical significance, we believe 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. There is substantial risk that the FDA, the EMA and other regulators 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 from the sales of Translarna for the treatment of nmDMD.

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If we do not successfully 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.

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

- whether the FDA, the EMA and other regulators agree with our interpretation of the results of ACT DMD, and the timelines within which such determinations are made;
- successful completion of our confirmatory Phase 3 ACT CF clinical trial of Translarna;
- our ability to successfully advance our submitted request for a marketing authorization variation with the EMA to seek inclusion of Translarna for the treatment of nmCF;
- the successful advancement of Translarna in additional indications, in particular, nmMPS I and nonsense mutation aniridia;
- the establishment of an expanded international commercial infrastructure capable of supporting product sales, marketing and distribution of Translarna;
- implementing marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- the continued maintenance of, and satisfaction of the conditions under, the marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area;
- 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;
- whether and when we obtain marketing approval of Translarna in additional territories and for additional or expanded indications;
- successful negotiation of adequate pricing and reimbursement terms for Translarna on a timely basis, or at all, in the countries which require such negotiation and in which we obtain regulatory approval;
- the timing and scope of the commercial launch of Translarna in nmDMD;
- establishing and maintaining commercial manufacturing arrangements with third party manufacturers;
- the ability of our third-party manufacturers to successfully produce commercial and clinical supplies of Translarna on a timely basis sufficient to meet the needs of our commercial and clinical activities;
- successful identification of eligible patients;
- acceptance of Translarna in nmDMD by patients, the medical community and third-party payors;

- effectively competing with other therapies;
- a continued acceptable safety profile of Translarna;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and
- protecting our rights in our intellectual property portfolio.

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

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The marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD is subject to the satisfaction of specific conditions and is limited to ambulatory patients aged five years and older located in the European Economic Area, which significantly limits an already small treatable patient population, reduces our commercial opportunities, and is subject to an annual reassessment of the risk-benefit balance by the EMA.

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. Overall, we estimate that the potential opportunity for a treatment for nmDMD is approximately 7,000 patients worldwide including 2,000 patients in the United States, 2,500 patients in the European Union and 2,500 patients in the rest-of-world including Latin America, Japan and Australia. We estimate that approximately 40% of nmDMD patients are ambulatory and at least five years old. 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, are based on our beliefs and estimates derived from a variety of sources and may prove to be incorrect. 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.

In addition, the marketing authorization granted by the European Commission is subject to annual review and renewal by the EMA following its reassessment of the risk-benefit balance of the authorization, which we refer to as the annul EMA reassessment, and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD in 2015, and our ability to implement measures, including pharmacovigilance plans, that are detailed in the risk management plan. We received EC approval of the annual renewal in August 2015. We plan to seek to renew the marketing authorization on an annual basis until we have satisfied the conditions of the marketing authorization and a full marketing authorization is granted. If we fail to satisfy such requirements, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require additional clinical trials, any of which would negatively impact our anticipated revenue from Translarna and materially harm our business. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance.

If clinical trials of our product or product candidates, such as our confirmatory Phase 3 clinical trials of Translarna, fail to demonstrate safety and efficacy to the satisfaction of the EMA or FDA, 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 approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

For example, the primary efficacy endpoint in the ITT population did not achieve statistical significance in the Phase 2b (completed in 2010) or Phase 3 ACT DMD (completed in 2015) clinical trials of Translarna for the treatment of nmDMD. Although we believe that the collective data from these trials, including pre-specified meta-analysis, pre-specified subgroup analyses and retrospective subgroup analyses that we have performed, provide strong support for concluding that Translarna was active and showed clinically meaningful improvements over placebo in these trials there is substantial risk that the FDA, the EMA and other regulators will not agree with our interpretation of the results of ACT DMD and the totality of clinical data from our trials.

In addition, the primary efficacy endpoint in the ITT population did not achieve statistical significance in our prior Phase 3 clinical trial of Translarna for the treatment of nmCF (completed in 2011) and did not achieve the primary objective in one of four prior Phase 2 clinical trials that we conducted for Translarna for the treatment of nmCF in which we measured change in chloride conductance in

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nasal cells over the course of treatment. We may similarly fail to achieve the primary efficacy endpoint in ACT CF, our confirmatory Phase 3 clinical trial of Translarna.

If the results of ACT CF are not favorable, or if the totality of data from our clinical trials in Translarna for the treatment of nmDMD fail to demonstrate safety and efficacy to the satisfaction of the FDA, the EMA or other regulators,we may need to conduct additional clinical trials at significant cost or altogether abandon development of Translarna for either or both of nmDMD and nmCF.

Further, the marketing authorization granted by the European Commission is subject to an annual reassessment by the EMA and is further conditioned on our submission of the final report, including additional efficacy and safety data from ACT DMD, during 2015. If the EMA does not view the results of ACT DMD as favorable, if we fail to satisfy the conditions of the marketing authorization, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require additional clinical trials. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance. We also sell Translarna under reimbursed early access programs in a limited number of countries and there is no assurance that such sales will continue to be permitted in any particular country. If any of these events were to occur, they would negatively impact our anticipated revenue from Translarna and would materially harm our business, financial results 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 related analyses); 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:

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

If we or our collaborators experience any of a number of possible unforeseen events in connection with clinical trials related to our product or product candidates, including those under our collaboration with Roche and the SMA Foundation, potential marketing approval or commercialization of our product or 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 approval or commercialize our product or product candidates, including:

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

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- 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 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 our product or 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 our product or product candidates may be greater than we anticipate;
- the supply or quality of our product or product candidates or other materials necessary to conduct clinical trials of our product or product candidates may be insufficient or inadequate; or

- our product or 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, we and our collaboration partners recently became aware of data from a 39-week non-clinical safety and toxicology study of RG7800 in cynomolgus monkeys, which showed an eye finding at exposures above those explored in SMA patients and healthy volunteers. As a precautionary measure, the collaboration partners decided in April 2015 to suspend dosing of additional patients to evaluate this finding and confirm next steps for the Phase 2 MOONFISH study. As a result, we and our collaboration partners may need to perform additional studies, may require consent of regulatory authorities, the re-initiation of the Phase 2 clinical study may be delayed or not allowed, or the trial's scope may be narrowed.

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

Our conclusions regarding the activity and potential efficacy of Translarna in nmDMD are primarily based on pre-specified meta-analysis and subgroup analyses of ACT DMD data and retrospective analyses of the results of our Phase 2b clinical trial of Translarna for the treatment of nmDMD. In addition, our conclusions regarding the activity and potential efficacy of Translarna in nmCF are primarily based on retrospective analyses of the results of our completed Phase 3 clinical trial of Translarna for nmCF. Other than pre-specified 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.

In October 2015, we announced the initial results of ACT DMD, including that the primary efficacy endpoint in the ITT population did not achieve statistical significance. However, consistent with the statistical analysis plan we submitted to the FDA for ACT DMD, we performed pre-specified subgroup analyses as well as pre-specified meta-analysis of ACT DMD data and data from the Phase 2b ambulatory decline phase subgroup. We believe that the results of these pre-specified analyses demonstrate that Translarna provides a meaningful clinical benefit for the treatment of nonsense mutation DMD.

The meta-analysis results, which favored Translarna in the 6MWT and each secondary end point timed function test, are considered statistically significant. 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. The p-values for the 6MWT and each secondary end point timed function test in the pre-specified subgroup of ACT DMD patients with a baseline 300-400 meter 6MWD also had p-values of less than 0.05, but due to the sequential testing method,

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these p-values are considered nominal. 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. 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.

In addition, after determining that the primary efficacy endpoint did not achieve statistical significance in our completed Phase 2b clinical trial of Translarna for the treatment of nmDMD and in our completed Phase 3 clinical trial of Translarna for nmCF, 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.

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. Our reliance on nominal p-values for some of our statistical data and our use of retrospective analyses could have a negative impact on whether the FDA, the EMA and other regulators agree with our interpretation of the results of ACT DMD and the timelines within which such determinations are made. In addition, it diminishes the likelihood that the EMA will approve our request of a variation to our marketing authorization for Translarna to include Translarna for nmCF on a conditional basis, could negatively impact the evaluation by the EMA or the FDA of our anticipated applications for full marketing approval for Translarna for nmDMD and the annual EMA reassessment process of our current marketing authorization, and, even if we successfully complete ACT CF, could negatively impact the evaluation by the EMA or the FDA of our anticipated applications for full marketing approval for Translarna for nmCF. If any of these events were to occur, they would negatively impact our anticipated revenue from Translarna and would materially harm our business, financial results and results of operations.

The results of ACT DMD and, even if successfully completed, the results of ACT CF, may not be sufficient for approval of Translarna for the applicable indication.

There is substantial risk that the FDA, the EMA and other regulators will not agree with our interpretation of the results of ACT DMD and the totality of data from our clinical trials of Translarna for the treatment of nmDMD and it is possible that, once completed and even if successful, the EMA or the FDA may not consider the results of ACT CF to be sufficient for approval of Translarna for such indication. The FDA typically requires two adequate and well-controlled pivotal clinical trials to support marketing approval of a product candidate for a particular indication. The EMA or the FDA could determine that the results of our trials are not sufficiently robust, are subject to confounding factors or are not adequately supported by other trial endpoints. In addition, although we have had discussions with the FDA regarding ACT CF, our confirmatory Phase 3 clinical trial of Translarna for the treatment of nmCF, the FDA may not consider our proposed trial design acceptable. For example, in 2012, the FDA indicated that in its view the data from our completed Phase 3 clinical trial and other data from our development program in cystic fibrosis do not by themselves support an NDA submission and, consequently, the FDA informed us that additional clinical data would be required to establish the evidence necessary to support eventual filing of an NDA for the use of Translarna to treat nmCF. We had additional interactions with the FDA in 2013 regarding the clinical development design which would have the potential to support an NDA, but we did not achieve a consensus between the EMA and FDA views. While we have incorporated feedback from the FDA into our ACT CF trial design, we believe that certain key recommendations from the FDA are not appropriate. Two of the key recommendations that we are in disagreement with are the

designation of FEV_1, CF pulmonary exacerbations and body mass index as three co-primary endpoints for the trial and a suggested three-year trial duration. FEV_1 is the primary endpoint in ACT CF, with CF pulmonary exacerbations and body mass index key secondary endpoints, which is consistent with other clinical trials currently ongoing in cystic fibrosis and FDA's earlier recommendation. Additionally, we believe that extending the study duration to three years would result in a number of complications that would ultimately limit the robustness of the data and conclusions that could be drawn from the results. Based on these interactions, we nonetheless initiated ACT CF in the first half of 2014 consistent with feedback from the EMA on our trial design. If the FDA does not consider our trial designs acceptable, we may need to conduct more than one confirmatory clinical trial and our ability to receive marketing approval for this indication could be delayed or prevented.

Because we are developing our product 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 or nmCF. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat either of these diseases. As a result, the design and

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conduct of clinical trials for these diseases, particularly for drugs to address the underlying nonsense mutations causing these diseases in some subsets of patients, is subject to increased risk.

Prior to 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.

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

We are faced with similar challenges in connection with the design of our Phase 2 proof-of-concept studies of Translarna in nmMPS I and aniridia caused by nonsense mutation because there is also limited historical clinical trial experience for the development of drugs to treat the underlying cause of these disorders. For example, with respect to nmMPS I, while clinical trials of enzyme replacement therapies conducted by third party sponsors have provided some insight into the disorder, enzyme replacement therapies do not sufficiently address the central nervous system, skeletal or cardiac symptoms associated with the disorder. In addition, our own pre-clinical and early stage clinical trials targeting nmMPS I have been limited in duration and, as a result, it is substantially uncertain whether our clinical design will optimize the duration or level of dosing or that we will be able to demonstrate a statistically significant biochemical or clinical effect in the primary or secondary pre-specified endpoints selected for the study.

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

We may not be able to initiate or continue clinical trials for our product candidates, including our confirmatory Phase 3 ACT CF clinical trial of Translarna or our Phase 2 proof-of-concept study of Translarna in nmMPS I or aniridia caused by nonsense mutation, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. For example, nmCF, nmMPS I and aniridia caused by nonsense mutation are characterized by relatively small patient populations, which could result in slow enrollment of clinical trial participants. In addition, our competitors have ongoing clinical trials for product candidates that could be competitive with our product candidates. 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 risks and benefits of the product candidate under study;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

Enrollment delays in our clinical trials may result in increased development costs for our product candidates. Our inability to enroll a sufficient number of patients in our confirmatory Phase 3 clinical trial of Translarna in nmCF or our Phase 2 proof-of-concept studies of Translarna in nmMPS I and aniridia caused by nonsense mutation or any of our other clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

In addition, during the third quarter of 2015 we submitted a request for a variation to our marketing authorization for Translarna in the EEA to seek approval for the treatment of nmCF. Although we submitted the variation request with the clinical results achieved in our prior Phase 3 clinical trial in nmCF, we believe that the CHMP will consider the status of ACT CF enrollment an important factor when considering our submission. In addition, we expect that even if the EMA approves a variance to include Translarna for nmCF, the EMA will require us, as a post-approval measure, to provide it with comprehensive clinical data from ACT CF. In addition, unless and until we satisfy the conditions of our primary marketing authorization in the EEA for Translarna, such authorization would continue to be subject to annual review and renewal by the EMA. See "Risk Factors—Risks Related to Regulatory Approval of our Product and our Product Candidates" on page 61 for further detail regarding the annual EMA reassessment process, including a description of the risk benefit balance. As a result, ACT CF enrollment delays could have a negative impact on our ability to obtain and maintain any marketing authorization for nmCF that may be granted in the future, if any.

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

Our product 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 our product or product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound.

For example, although we did not observe a pattern of liver enzyme elevations in our Phase 2 or Phase 3 clinical trials of 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 our completed 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. If patients in the Translarna arm of a confirmatory Phase 3 clinical trial for the treatment of nmCF exhibit clinically meaningful creatinine elevations, the EMA or the FDA might not approve Translarna for this indication or could require that we instruct physicians to frequently monitor patients for these abnormalities or impose other conditions, which may be an impediment to the use of Translarna because of concerns related to its safety and convenience.

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

Our scientific approach focuses on the discovery and development of product candidates that target post-transcriptional control processes. While a number of commonly used drugs and a growing body of research validate the importance of post-transcriptional control processes in the origin and progression of a number of diseases, no existing drugs have been specifically designed to alter post-transcriptional control processes in the same manner as 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. 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 annual EMA reassessment, and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD in 2015. We may not be successful in developing and receiving full regulatory approval for such use and we may not receive regulatory approval for additional indications for Translarna or 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, or any other product candidate that receives marketing approval, 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, Translarna and any of our other product candidates that may receive marketing approval may nonetheless fail to gain sufficient market acceptance by physicians, patients, third party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or any profits from operations. The degree of market acceptance of our product 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 product 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; and
- any restrictions on concomitant use of other medications, such as a restriction that nmCF patients taking Translarna not also use chronic inhaled aminoglycoside antibiotics.

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

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

We have limited experience in the sale and marketing of pharmaceutical products, and we may be unable to successfully execute our commercial strategy in the EEA or, if approved, in the United States or other territories. Our commercial strategy for Translarna involves the development of a commercial infrastructure that spans multiple jurisdictions. In preparation for a potential U.S. launch in the first half of 2016, we are evaluating our commercial strategy and have begun building out our commercial team and infrastructure in the United States Our ability to successfully commercialize Translarna for the treatment of nmDMD in the EEA and other territories, including the United States, if approved, is heavily dependent upon our ability to continue to build an infrastructure that is capable of implementing our global commercial strategy. International operations are subject to inherent risks. 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 in a timely manner or at all. Doing so will require a high degree of coordination and compliance with laws and regulations in numerous jurisdictions, including restrictions on advertising practices, enforcement of intellectual property rights, restrictions on pricing or discounts, and unexpected changes in international regulatory requirements and tariffs. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize Translarna in those jurisdictions in which it is or may be approved 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 have evaluated markets outside of the EEA to determine in which geographies we might, if approved, choose to commercialize Translarna ourselves and in which geographies we might choose to collaborate with third parties. We intend to continue to promote Translarna for the treatment of nmDMD in permitted territories using both internal and external resources. 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 our

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commercialization efforts for Translarna for the treatment of nmDMD or any other product launch. If the commercial launch of Translarna or any other 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 product candidates.

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

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to implement third party marketing and distribution relationships in territories where we do not pursue direct commercialization;
- the inability of our commercial team to obtain access to or persuade adequate numbers of physicians to prescribe Translarna 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.

We plan to develop our commercial strategy for additional indications for Translarna or other product candidates, if and when such drugs are approved in the applicable region.

We expect that during 2015 all of our sales of Translarna for the treatment of nmDMD will 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.

We expect that during 2015, all of our revenue from sales of Translarna will be generated from countries other than the United States. Additionally, we have operations in several European countries and other territories. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

- changes in international regulatory and compliance requirements 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;
- 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.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations. In addition, in some countries, including Brazil, orders for named patient sales are for multiple months of therapy, which can lead to an unevenness in orders which could result in significant fluctuations in quarterly net product sales. 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 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 Duchenne muscular dystrophy. Other currently available treatments for Duchenne muscular dystrophy are only palliative. However, there are other biopharmaceutical companies, including Santhera Pharmaceuticals, BioMarin Pharmaceutical Inc. (following its acquisition of Prosensa in early 2015) and Sarepta Therapeutics that are developing treatments for Duchenne muscular dystrophy. Summit Corporation also has a product candidate in early clinical development designed to increase the production of the protein utrophin, which is functionally similar to dystrophin, to treat Duchenne muscular dystrophy.

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

In addition, Aldurazyme, which is manufactured by BioMarin Pharmaceutical Inc. and sold by Genzyme Corporation, is an enzyme replacement therapy for the treatment of mucopolysaccharidosis I. Our SMA collaboration with Roche and the SMA Foundation also faces competition. For example, Isis Pharmaceuticals, Inc. is evaluating its antisense drug as a treatment for SMA in two Phase 3 studies that were initiated in SMA patients in 2014. Other companies are also pursuing product candidates for the treatment of SMA, including Novartis Pharmaceuticals Corporation.

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

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

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

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

We currently expect that Translarna will be priced at levels consistent with the pricing for other therapies for the treatment of rare disorders where high unmet medical need exists.

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

Our ability to commercialize Translarna or any other product candidate successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the EU and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Prices at which we or our customers seek reimbursement for our products 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 predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for Translarna or any other product that we commercialize and, if coverage and reimbursement are available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for Translarna may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

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

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

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

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- reduced resources of our management to pursue our business strategy;
- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- increased insurance costs, or an inability to maintain appropriate insurance coverage;

- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We have product liability insurance that covers our commercial sales, sales pursuant to reimbursed early access programs and clinical trials up to a $25.0 million annual aggregate limit, and subject to a per claim deductible. 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 continue commercializing Translarna or as and when we begin commercializing any other product candidate that receives marketing approval. The cost of insurance coverage is highly variable, based on a wide range of factors, and is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability 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 production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. For example, we initiated separate Phase 2 clinical trials of 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. 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.

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We have based our research and development efforts on small-molecule drugs that target post-transcriptional control processes. Notwithstanding our large investment to date and anticipated future expenditures in proprietary technologies, including GEMS and our alternative splicing technology, which we use in the discovery of these molecules, to date we have only been granted marketing authorization to treat nmDMD under a restricted label, and subject to the fulfillment of certain conditions, in the European Economic Area. We may not be able to satisfy the conditions of our current marketing authorization for nmDMD, including the submission of the results of ACT DMD to the EMA during 2015, 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 our Dependence on Third Parties

We contract with third parties for the manufacture and distribution of our product 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 product candidates. We have limited personnel with experience in drug manufacturing and lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on third parties for supply of the active pharmaceutical ingredients in Translarna 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.

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. In the first half of 2015, we engaged a third party logistics, or 3PL, provider in the European Union, which has commenced distribution of Translarna for the majority of our commercial and EAP programs on our behalf.

We currently rely on a single source for the production of some of our raw materials and we obtain our supply of the bulk drug substance for Translarna from two third-party manufacturers and the bulk drug substance for our antibacterial and cancer stem cell programs through another third-party manufacturer. We engage a separate manufacturer to provide bulk drug product and expect to finalize our validation of another bulk drug manufacturer in early 2016. We engage a separate manufacturer to provide fill and finish services for our finished commercial and clinical product and are in the process of completing arrangements with two additional providers to provide these services, initially for our clinical product, by the end of 2015, with commercial product services expected to commence for at least one provider in 2016. 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.

We may be unable to conclude agreements for commercial or clinical supply with third-party manufacturers, or may be unable to do so on acceptable terms.

Even if we are able to establish and maintain arrangements with third-party manufacturers and distributors, reliance on such service providers 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 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; 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 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, 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 or our product candidates.

Our product 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.

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 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 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 and our product candidates may adversely affect our business, financial condition, results of operations and growth including our ability to develop product candidates and commercialize our products that receive regulatory approval on a timely and competitive basis.

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

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

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

For example, in the first half of 2013 inspectors acting at the request of the EMA conducted GCP inspections of selected clinical sites from our completed Phase 2b clinical trial of Translarna for the treatment of nmDMD and our clinical trial site relating to our then pending MAA 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

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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 approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Our product development costs will increase if we experience delays in testing or obtaining marketing approvals.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approvals of our product 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 collaborator(s) 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;

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- 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.

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

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

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

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

Risks Related to our Intellectual Property

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

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States and in certain foreign jurisdictions related to our novel technologies, 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 patent protection in all major markets. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent

applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Moreover, we may be subject to a third party submission of prior art to a patent office or become involved in addressing patentability objections based on anonymous submission of references, opposition, derivation, reexamination, inter partes review, post grant review, interference proceedings or other patent office proceedings or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize 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 submitted as part of an MAA becoming publicly available. The EMA Policy on publication of clinical data, 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) that would normally be maintained by a regulatory body as confidential. Such developments could enable other companies to circumvent our intellectual property rights and use our clinical trial data to obtain marketing authorizations in the European Union and in other jurisdictions. Such developments may also require us to allocate significant resources 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.

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

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

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

Third parties may initiate legal proceedings alleging that 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 product 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

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intellectual property rights potentially relating to our product and our product candidates. For example, we have not conducted a recent freedom-to-operate search or analysis for Translarna™ (brand name of ataluren). Any freedom-to-operate search or analysis previously conducted may not have uncovered all relevant patents and patent applications, and there may be pending or future patent applications that, if issued, would block us from commercializing Translarna. Thus, we do not know with certainty whether Translarna, any other product candidate, or our commercialization thereof, does not and will not infringe any third party's intellectual property.

If we are found to infringe a third party's intellectual property rights, or in order to avoid or settle litigation, we could be required to obtain a license to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us, and could require us to make substantial payments. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from commercializing our product 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. 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 Translarna, even though neither the issued U.S. patent nor any of the international patents or patent applications specifically discloses Translarna. In order to successfully challenge the validity of any issued U.S. patent that may allegedly include Translarna within the scope of a granted claim, we would need to overcome a presumption of validity. This burden is a high one requiring us to present clear and convincing evidence as to the invalidity of these claims. There is no assurance that a court would find these claims to be invalid. In addition, we believe that our testing of Translarna in clinical trials for the purpose of seeking FDA approval would be a valid defense against any infringement claims in the United States based on the availability of a statutory exemption. However, there can be no assurance that our interpretation of the statutory exemption would be upheld, and the statutory exemption would only cover our preclinical research activities, and not the commercialization of Translarna.

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

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proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. Any party with whom we have executed such an agreement may

breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be obtained or independently developed by a competitor, our competitive position would be harmed.

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

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

If we are not able to obtain adequate trademark protection or regulatory approval for our brand names, including Translarna, 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 product or our product candidates, including Translarna, we may seek to register such trademarks. Trademark registration is territory-specific and we must apply for registration in the United States as well as any other country where we intend to commercialize product under a registered trademark. Failure to obtain the appropriate registrations may place our use of the trademark at risk or make it subject to legal challenges, which could force us to choose an alternative name for our product candidates. In addition, the FDA, and other regulatory authorities outside the United States, typically conduct a separate review of proposed product names for pharmaceuticals, including an evaluation of potential for confusion with other product names or medication or prescribing errors. These regulatory authorities may also object to any product name we submit if they believe the name inappropriately implies medical claims. If the FDA or other competent regulatory authority outside the United States objects to any of our proposed product names, we may be required to adopt an alternative name for our product candidates. If we adopt an alternative name, either because of our inability to obtain a trademark registration or approval or related legal challenges or because of objections from regulatory authorities, we would lose the benefit of our existing trademark applications for such product or product candidate, and may be required to expend significant additional resources in an effort to identify a suitable product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA or applicable other regulatory authority, which could cause delays in getting our products to market and substantially increase our costs. We may be unable 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.

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

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

Translarna 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 marketing approval for a product candidate will prevent us from commercializing the product candidate.

We announced the initial results of ACT DMD, our confirmatory Phase 3 trial in nmDMD, in October 2015. While the primary efficacy endpoint in the ITT population, did not achieve statistical significance in ACT DMD, we believe 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. There is substantial risk that the FDA, the EMA and other regulators 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 from the sales of Translarna for the treatment of nmDMD. See “Risk Factors - Risks Related to the Development and Commercialization of our Product and Product Candidates” on page 40 for further detail regarding how the ACT DMD results could impact our ability to commercialize Translarna.

We received marketing authorization to market Translarna for nmDMD in the European Economic Area in the third quarter of 2014. This marketing authorization is subject to annual EMA reassessment and is further conditioned on our submission of the final report, including additional efficacy and safety data, from our ACT DMD during 2015. We have not otherwise received marketing approval for Translarna or any of our other product candidates from regulatory authorities in any jurisdiction.

We have begun seeking and intend to continue to seek marketing approval for Translarna for the treatment of nmDMD in territories outside of the EEA. In December 2014, we commenced a rolling basis submission of a new drug application, or NDA, to the FDA for approval of Translarna for the treatment of nmDMD. We expect that the submission of our ACT DMD data will complete our rolling NDA and allow for FDA review. We may not receive necessary approvals from the FDA or other regulators to further commercialize Translarna or any product candidate in any market.

In addition, during the third quarter of 2015 we submitted a variation to our marketing authorization application with the EMA to seek to include Translarna for the treatment of nmCF. There is substantial risk that the EMA will not grant us approval of Translarna for the treatment of nmCF. We expect that even if the EMA approves a variance to include Translarna for nmCF, the EMA will require us, as a post-approval measure, to provide it with comprehensive clinical data from ACT CF. In addition, unless and until we satisfy the conditions of our primary marketing authorization in the EEA for Translarna, such authorization will continue to be subject to annual review and renewal by the EMA.

We have only limited experience in filing and supporting the applications necessary to obtain marketing approvals for product candidates and expect to continue to rely on third-party contract research organizations to assist us in this process. Securing marketing approval 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 approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that 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 approval or that prevent or limit commercial use.

The process of obtaining marketing approvals is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

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For example, our ability to obtain and maintain marketing authorizations granted on a conditional basis in the European Economic Area is limited to specific circumstances and subject to several conditions and obligations. Conditional marketing authorizations based on incomplete clinical data, including our marketing authorization for Translarna for the treatment of nmDMD, may be granted 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 risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) unmet medical needs will be fulfilled and (4) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations, including with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data, may be specified in the conditional marketing authorization. Marketing authorizations subject to conditions are only valid for one year, and must be renewed annually by the EMA after an assessment of the risk-benefit balance and need for additional or modified conditions.

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

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

Regulatory authorities in some jurisdictions, including 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, nmCF, nmMPS I, and nonsense mutation aniridia. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the EMA from accepting another marketing application for a similar medicinal product or the FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable market exclusivity period is 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 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 orphan drug exclusivity for and approval of a product 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. 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, 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.

The fast track designation for Translarna may not actually lead to a faster development or regulatory review or approval process and we may be unable to secure clearances from the FDA or comparable foreign regulatory authorities to use an expedited development, review or approval pathway.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. We have obtained a fast track designation from the FDA for Translarna for the treatment of nmDMD. However, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw our fast track designation if the FDA believes that the designation is no longer supported by data from our clinical development program. Our fast track designation does not guarantee that we will qualify for or be able to take advantage of the FDA’s expedited review procedures, nor does it increase the likelihood of FDA approval. Similarly, while we anticipate that we will pursue expedited development, review or approval pathways

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for Translarna and future product candidates, we may be unable to secure clearance to follow any such expedited pathway from the FDA or comparable foreign regulatory authorities. In addition, although access to an expedited program may expedite the development, review or approval process, it does not change the standards for approval and qualification for any expedited review procedure does not ensure that we will obtain regulatory approval for Translarna or any other product candidate that we may develop.

All pharmaceutical products for which marketing authorization has been granted, including Translarna for the treatment of nmDMD, 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.

The company, Translarna, 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 approval. Any regulatory approval of any of our products or product candidates, once obtained, may be withdrawn. For example, our marketing authorization in the EEA is subject to annual EMA reassessment and is further conditioned on our submission of the final report, including additional efficacy and safety data, from ACT DMD during 2015. 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.

We are required to submit safety and other post-market information and reports, implement pharmacovigilance plans, and comply with 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 authorization for Translarna for the treatment of nmDMD 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.

Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval, including the requirement of risk evaluation and mitigation strategy, or REMS. 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 manufacturers' communications regarding off-label use and if we do not comply with the laws governing promotion of approved drugs, we may be subject to enforcement action for off-label promotion. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to 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;

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- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions;

- the imposition of civil or criminal penalties; or
- debarment.

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

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.

Our initial commercial launch of Translarna has begun in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues, if any. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna in the European Economic Area and other jurisdictions would prevent us from marketing our products in such regions.

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 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. For example, since December 2014, Translarna for the treatment of nmDMD has been commercially available in Germany, but we are still in the process of engaging with relevant German authorities to finalize pricing in that country. 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 key EEA countries, we have not received both pricing and reimbursement approval in any country, and there is no assurance that we will receive such approval or, if we do, that the price, level of reimbursement and other terms will be acceptable to us. In addition, the price that is approved by local governmental authorities pursuant to commercial pricing and reimbursement processes may be significantly lower than the price we are able to charge for sales under our reimbursed early access

programs. In some instances, reimbursement may be subject to challenge, reduction or denial by the government and other payers. In some countries, such as 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, the company 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.

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, during the fourth quarter of 2014, the publicly funded health care system in the UK determined to reconsider how it assesses certain new treatments and postponed certain pricing and reimbursement meetings, including meetings related to Translarna, and in July 2015 determined that final funding decisions on Translarna for nmDMD will be made after the conclusion of a specialized appraisal process. During the fourth quarter of 2015, following our first meeting with the UK health care system, draft guidance was published by the health care system noting that it was minded to say no to Translarna reimbursement for the treatment of nmDMD, subject to our response to a request for additional information and a follow-on committee meeting expected in the fourth quarter of 2015. We expect final guidance to follow in the first quarter of 2016.

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. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries.

If we fail to successfully secure and maintain pricing and reimbursement coverage for Translarna or are significantly delayed in doing so or if burdensome conditions are imposed by private payers, government authorities or other third-party payers on such reimbursement, planned launches in the affected countries will be delayed and our anticipated revenue and growth prospects could be negatively affected and our business could be adversely affected.

Our relationships with customers, healthcare providers and professionals 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. 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 approval.

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 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 federal and state healthcare laws and regulations, and equivalent laws and regulations in the European Union, include, and are not limited to, the following:

- Anti-corruption and anti-bribery statutes, including the U.S. Foreign Corrupt Practices Act, or FCPA, and the UK Bribery Act of 2010, or Bribery Act. These statutes are generally broad in scope and will require us to make and keep books and records that accurately and fairly reflect the transactions of the company and to devise and maintain an adequate system of internal accounting controls. The FCPA prohibits the offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-U.S. government official in order to improperly influence any act or decision, secure any other improper advantage, or obtain or retain business. Under the UK Bribery Act, companies which carry on a business or part of a business in the United Kingdom may be held liable for bribes given, offered or promised to any person, including non-UK government officials and private persons, by employees and persons associated with the company in order to obtain or retain business or a business advantage for the company. Other countries have adopted, or may adopt in the future, similar anti-corruption and anti-briberty statutes with which we may be required to comply.

- Anti-kickback statutes, 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 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 commercial payors. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse, and healthcare laws and regulations.

- The Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively the Affordable Care Act), amended the intent requirement of the federal anti-kickback statute such that a person no longer needs to have actual knowledge of the statue or specific intent to violate it.

- Laws and regulations, including the U.S. False Claims Act, which impose civil and criminal liability on individuals and entities who submit, or cause to be submitted, false or fraudulent claims for payment to the government. The U.S. government has brought False Claims Act actions against pharmaceutical companies on the theory that their practices have caused false claims to be submitted to the government. The U.S. Attorneys' Offices and the main Department of Justice have taken broad interpretations of what constitutes falsity or false claims. A wide range of pharmaceutical manufacturers' commercial activity, marketing practices and price reporting practices have been scrutinized as potential violations of the False Claims Act.

- The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. 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 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 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 regulating off-label promotion. Off-label promotion of medicinal products 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. Under the Federal Food, Drug and Cosmetic Act and other laws, if any of our product candidates are approved, we would be prohibited from promoting our products for off-label uses. This means, for example, that we would not be able to make claims about the use of our marketed products outside of their approved indications, and we would not be able to proactively discuss or provide information on off-label uses of such products, with very specific and limited exceptions. The FDA does not, however, restrict physicians from prescribing products for off-label uses in the practice of medicine. 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. In addition, failure to follow FDA rules and guidelines relating to promotion and advertising can result in, among other things, the FDA's refusal to approve a product, the suspension or withdrawal of an approved product from the market, product recalls, fines, disgorgement of money, operating restrictions, injunctions or criminal prosecutions.

- Statutory requirements to disclose publicly payments made to physicians, including in certain EU member states and the United States. For example, under federal Physician 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, and physician ownership and investment interests in such manufacturers. A number of states have enacted their own transparency requirements that obligate manufacturers to report different types of spending related to physicians and other covered recipients.

- Laws 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

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information to physicians concerning the safe and effective use of the medicinal product. Promotion of indications not covered by the SmPC is specifically prohibited.

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

In addition, interactions between pharmaceutical companies and physicians are also governed by industry self-regulation codes of conduct and physicians' codes of professional conduct. 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 will involve substantial costs. We cannot guarantee that we, our employees, our consultants or our third-party contractors are or will be in compliance with all federal, state and foreign regulations and codes. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, and the curtailment or restructuring of our operations. Exclusion, suspension and debarment from government funded healthcare programs would significantly impact our ability to commercialize, sell or distribute any drug. Even if we are 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. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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

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

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

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

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There have been multiple attempts through legislative action and legal challenge to repeal or amend the Affordable Care Act. Although the U.S. Supreme Court in King v. Burwell upheld the use of subsidies to individuals in federally facilitated health care exchanges on June 25, 2015, which ultimately did not disrupt significantly the implementation of the Affordable Care Act, we cannot predict whether other current or future efforts to repeal or amend these laws will be successful, nor can we predict the impact that such a repeal or amendment would have on our business and operations, or on our results of operations. 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 addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, the Budget Control Act of 2011, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, which triggered the legislation's automatic reduction to several government programs, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which delayed for another two months the budget cuts mandated by the sequestration provisions of the Budget Control Act of 2011. The ATRA, among other things, also reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In March 2013, the President signed an executive order implementing sequestration, and in April 2013, the 2% Medicare reductions

went into effect. In December 2013, Congress amended the Budget Control Act to provide greater discretionary spending in 2014 and 2015 than originally budgeted and provide relief from the FDA user fee for two years; this legislation also extended the prohibition against reducing payments to Medicare providers by more than 2% for two years (i.e., until 2023). In December 2014, Congress passed an omnibus funding bill (the Consolidated and Further Continuing Appropriations Act, 2015) and a tax extenders bill, both of which may negatively impact coverage and reimbursement of healthcare items and services.

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

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize 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.

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.

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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, we have experienced and plan 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 and will continue to impose significant added responsibilities on members of management, including the need to recruit, hire, retain, motivate and integrate additional employees. 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. 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. 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 3.00% convertible senior notes due August 15, 2022, or 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

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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.

An active trading market for our common stock may not be sustained.

Our shares of common stock began trading on The NASDAQ Global Select Market on June 20, 2013. Given the limited trading history of our common stock, there is a risk that an active trading market for our common stock will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

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

Our stock price may be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, 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:

- our ability to complete our regulatory submissions in a timely manner, including our rolling NDA with the FDA and our final report with respect to ACT DMD to the EMA for Translarna for the treatment of nmDMD;
- whether the FDA, the EMA and 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;
- results of clinical trials of Translarna and any other product candidate that we develop;
- 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;

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- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

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

We are an "emerging growth company", as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. Because the market value of our common stock that is held by non-affiliates exceeded $700 million as of June 30, 2015, we will cease to be an emerging growth company as of December 31, 2015.

As an emerging growth company, we are permitted and have relied upon exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include: not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting; not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements; reduced disclosure obligations regarding executive compensation; and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

Until we no longer qualify as an emerging growth company, we may choose to take advantage of some, but not all, of the available exemptions. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

We 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, and particularly after we are no longer an "emerging growth 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

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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. As an emerging growth company, we have not been required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, we expect that we will be required to file such an attestation report on internal control over financial reporting issued by our independent registered public accounting firm with respect to the year ending December 31, 2015. To achieve compliance with Section 404 within the prescribed period, we have been engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we have continued to dedicate internal resources, engage outside consultants and

adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. 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. A significant number of our shares are currently "restricted" securities as a result of securities laws, but are able to be sold, subject to any applicable volume limitations under federal laws with respect to affiliate sales. Moreover, certain holders of an aggregate of 764,036 shares of our common stock have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

We have issued a significant number of restricted stock awards and shares that are subject to outstanding options 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 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.

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, 2015, we issued options to purchase an aggregate of 166,200 shares of common stock to certain new hire employees at a weighted-

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average exercise price of $35.97 per share. The shares underlying these option awards will be registered on a Form S-8 registration statement prior to the first vesting event applicable to each such award.

Item 6. Exhibits.

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

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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 9, 2015

By: /s/ Shane Kovacs
Shane Kovacs
Chief Financial Officer
(Principal Financial and Accounting Officer and Duly Authorized Signatory)

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EXHIBIT INDEX

Exhibit Number	Description of Exhibit
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.

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 9, 2015

By: /s/ STUART W. PELTZ
Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Shane Kovacs, 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 9, 2015

By: /s/ SHANE KOVACS
Shane Kovacs
Chief 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, 2015 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 9, 2015

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, 2015 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Shane Kovacs, Chief 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 9, 2015

By: /s/ SHANE KOVACS
Shane Kovacs
Chief Financial Officer
(Principal Financial and Accounting Officer)