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 June 30, 2014

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 o
Non-accelerated filer x (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 August 4, 2014 there were 30,069,897 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 Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate", "believe", "estimate", "expect", "intend", "may", "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 and conduct of our clinical trials of Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy, cystic fibrosis and mucopolysaccharidosis type I, or MPS I, caused by nonsense mutations, as well as our trials in spinal muscular atrophy and BMI1, 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;
- our plans to pursue development of Translarna for additional indications other than Duchenne muscular dystrophy, cystic fibrosis and MPS I, caused by nonsense mutations;
- 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;
- the rate and degree of market acceptance and clinical utility of Translarna;
- our ability to maintain the conditional marketing authorization of Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in the European Economic Area;
- the timing of and our ability to obtain additional marketing approvals of Translarna and our other product candidates, and the ability of Translarna and our other product candidates to meet existing or future regulatory standards;
- 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;
- our ability to commercialize Translarna in general, and specifically as a treatment for nmDMD, including our ability to successfully negotiate favorable pricing and reimbursement processes in the countries in which we may obtain regulatory approval;
- 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 our 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;
- 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 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

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- our expectations with respect to the development and regulatory status of our 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, 2013 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

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

	June 30, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 21,172	$ 15,414
Marketable securities	205,687	127,053
Prepaid expenses and other current assets	2,597	1,599
Grant and collaboration receivables, net	858	958
Total current assets	230,314	145,024
Fixed assets, net	6,417	6,730
Deposits and other assets	827	149
Total assets	$ 237,558	$ 151,903
Liabilities and stockholders’ equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 11,341	$ 12,207
Current portion of long-term debt	—	49
Deferred revenue	242	878
Total current liabilities	11,583	13,134
Other long-term liabilities	2,219	2,227
Total liabilities	13,802	15,361
Stockholders’ equity:		
Preferred stock, $0.001 par value. Undesignated 5,000,000 shares; issued and outstanding 0 shares at June 30, 2014 and December 31, 2013	—	—
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 29,340,577 shares at June 30, 2014. Authorized 125,000,000 shares; issued and outstanding 23,803,282 shares at December 31, 2013	30	24
Additional paid-in capital	591,636	465,246
Accumulated other comprehensive income	90	70
Accumulated deficit	(368,000)	(328,798)
Total stockholders’ equity	223,756	136,542
Total liabilities and stockholders’ equity	$ 237,558	$ 151,903

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Revenues:				
Collaboration revenue	$ 1,418	$ 5,868	$ 10,565	$ 11,940
Grant revenue	259	986	329	2,056
Total revenues	1,677	6,854	10,894	13,996
Operating expenses:				
Research and development	18,313	14,712	34,202	25,969
General and administrative	8,733	6,595	16,273	11,056
Total operating expenses	27,046	21,307	50,475	37,025
Loss from operations	(25,369)	(14,453)	(39,581)	(23,029)
Interest income (expense), net	248	(114)	419	(6,276)
Other income (expense), net	17	(19)	(40)	34
Net loss	(25,104)	(14,586)	(39,202)	(29,271)
Deemed dividend	—	—	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	—	3,391
Net loss attributable to common stockholders	$ (25,104)	$ (14,586)	$ (39,202)	$ (44,129)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	29,332,227	2,648,832	27,976,847	1,326,679
Net loss per share applicable to common stockholders—basic and diluted (in dollars per share)	$ (0.86)	$ (5.51)	$ (1.40)	$ (33.26)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Statements of comprehensive loss (unaudited)
In thousands

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Net loss	$ (25,104)	$ (14,586)	$ (39,202)	$ (29,271)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	10	(1)	20	(1)
Comprehensive loss	$ (25,094)	$ (14,587)	$ (39,182)	$ (29,272)

See accompanying unaudited notes.

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

	Six months ended June 30,	
	2014	2013
Cash flows from operating activities		
Net loss	$ (39,202)	$ (29,271)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	1,150	1,222
Change in valuation of warrant liability	38	(34)
Non-cash interest expense	—	6,044
Amortization of premiums on investments	910	—
Share-based compensation expense	7,983	2,502
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(998)	(760)

Grant and collaboration receivables	100	143
Deposits and other assets	(678)	41
Accounts payable and accrued expenses	(866)	3,035
Other long-term liabilities	(46)	3
Deferred revenue	(636)	(10,207)
Net cash used in operating activities	(32,245)	(27,282)
Cash flows from investing activities		
Purchases of fixed assets	(837)	(96)
Purchases of marketable securities	(132,603)	(11,604)
Maturities of marketable securities	53,079	20
Net cash used in investing activities	(80,361)	(11,680)
Cash flows from financing activities		
Payments on long-term debt	(49)	(2,174)
Net proceeds from sale of Series Four convertible preferred stock	—	60,785
Proceeds from exercise of options	30	—
Net proceeds from public offerings	118,383	131,720
Net cash provided by financing activities	118,364	190,331
Net increase in cash and cash equivalents	5,758	151,369
Cash and cash equivalents, beginning of period	15,414	2,726
Cash and cash equivalents, end of period	$ 21,172	$ 154,095
Supplemental disclosure of cash information		
Cash paid for interest	$ 1	$ 282
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized gain (loss) on marketable securities	$ 20	$ (1)
Change in carry value of preferred securities resulting from recapitalization	$ —	$ 3,391
IPO closing costs included in accounts payable and accrued expenses	$ —	$ 1,730

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Notes to unaudited financial statements
June 30, 2014
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. During the second quarter of 2014, a wholly-owned subsidiary was established in Bermuda to hold certain intellectual property rights of the Company. In addition wholly-owned subsidiaries in Ireland and in Denmark were established during the second and third quarter of 2014, respectively. PTC is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. The Company's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that PTC applies in our drug discovery activities and in collaborations with leading biopharmaceutical companies.

The Company's lead candidate is ataluren, an investigational new drug in the US, for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. The brand name of ataluren is Translarna™. On August 4, 2014, the Company was notified that the European Commission, or EC, granted conditional marketing authorization for Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The conditional marketing authorization allows the Company to market Translarna in the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland and Liechtenstein. The conditional marketing authorization is subject to an annual review by the EMA and the Company will seek to renew the approval on an annual basis until its obligations have been fulfilled and the approval is converted from a conditional approval into a full approval.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of June 30, 2014, the Company had an accumulated deficit of approximately $368.0 million. The Company has financed its operations to date primarily through a public offering of common stock in February 2014, its initial public offering of common stock in June 2013 (see note 6 below), 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.

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, 2013 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 6, 2014 (2013 Form 10-K). There have been no changes to our accounting policies during the quarter.

Basis of Presentation

The accompanying unaudited financial information as of June 30, 2014 and for the three and six months ended June 30, 2014 and 2013 has been prepared by the Company 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, 2013 and notes thereto included in the 2013 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2014 and for the three and six months ended June 30, 2014 and 2013 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three and six month periods ended June 30, 2014 are not necessarily indicative of the results to be expected for the year ended December 31, 2014 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.

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Recently issued accounting standard

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. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2016. Early application is not permitted. Entities can transition to the standard either retrospectively or as a cumulative-effect adjustment as of the date of adoption. Presently, the Company is assessing what effect the adoption of ASU 2014-09 will have on its financial statements and accompanying notes.

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 above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of June 30, 2014 and December 31, 2013:

	June 30, 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	$ 205,687	$ —	$ 205,687	$ —
Warrant liability	96	—	—	96

	December 31, 2013			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 127,053	$ —	$ 127,053	$ —

Warrant Liability	58	—	—	58

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The following is a summary of marketable securities accounted for as available-for-sale securities at June 30, 2014 and December 31, 2013:

	June 30, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Corporate debt securities	$ 166,564	$ 185	$ (77)	$ 166,672
Government obligations	39,033	5	(23)	39,015
	$ 205,597	$ 190	$ (100)	$ 205,687

	December 31, 2013			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 14,993	$ 5	$ —	$ 14,998
Corporate debt securities	111,989	97	(31)	112,055
	$ 126,982	$ 102	$ (31)	$ 127,053

At June 30, 2014 and December 31, 2013, 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 June 30, 2014 and December 31, 2013 mature as follows:

	June 30, 2014	
	Less Than 12 Months	More Than 12 Months
Corporate debt securities	$ 71,387	$ 95,285
Government obligations	—	39,015
Total Marketable securities	$ 71,387	$ 134,300

	December 31, 2013	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 14,998	$ —
Corporate debt securities	54,159	57,896
Total Marketable securities	$ 69,157	$ 57,896

Level 3 valuation

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

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuation for warrant liability for the period ended June 30, 2014:

	Level 3 assets
Beginning balance as of December 31, 2013	$ 58
Change in fair value of warrant liability	38
Ending balance as of June 30, 2014	$ 96

Fair value of the warrant liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of June 30, 2014 include (i) volatility (81%—83%), (ii) risk free interest rate (0.88%—1.62%), (iii) strike price ($128.00), (iv) fair value of common stock ($26.14), and (v) expected life

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(2.96—5.23 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2013 include (i) volatility (61-89%), (ii) risk free interest rate (0.07%—2.10%), (iii) strike price ($128.00—$2,520.00), (iv) fair value of common stock ($16.97), and (v) expected life (0.30—5.70 years). See Note 6 for a description of the warrants issued in connection with the convertible notes.

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

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

The following table summarizes other comprehensive income and the changes in accumulated other comprehensive items for the three months ended June 30, 2014:

	Unrealized Gains On Marketable Securities	Total Accumulated Other Comprehensive Items
Balance at March 31, 2014	$ 80	$ 80
Other comprehensive income before reclassifications	10	10
Amounts reclassified from other comprehensive items	—	—
Other comprehensive income	10	10
Balance at June 30, 2014	$ 90	$ 90

The following table summarizes other comprehensive income and the changes in accumulated other comprehensive items for the six months ended June 30, 2014:

	Unrealized Gains On Marketable Securities	Total Accumulated Other Comprehensive Items
Balance at December 31, 2013	$ 70	$ 70
Other comprehensive income before reclassifications	20	20
Amounts reclassified from other comprehensive items	—	—
Other comprehensive income	20	20
Balance at June 30, 2014	$ 90	$ 90

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at June 30, 2014 and December 31, 2013 consist of the following:

	June 30, 2014	December 31, 2013
Employee compensation, benefits, and related accruals	$ 4,149	$ 5,103
Consulting and contracted research	3,851	4,006
Professional fees	1,814	1,294
Accounts payable	613	1,124
Other	914	680
	$ 11,341	$ 12,207

6. Capital structure

2013 Recapitalization

During January and February of 2013, the Company entered into a “bridge” financing arrangement with certain existing investors providing for the issuance by the Company of an aggregate of $6 million of convertible promissory notes and warrants to purchase 2,527,675 shares of Series One convertible preferred stock (Series One) and Series Two convertible preferred stock (Series Two). The warrants have a per share exercise price of $0.01, and as such, they are referred to as “penny warrants”. This bridge financing was closed in anticipation of the March 2013 Series Four financing event, which the Company refers to as the “2013 recapitalization”.

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The Company allocated the proceeds of the convertible promissory notes between debt and warrant liability. Since the value of the warrants exceeded the proceeds from the convertible notes issued to existing investors, the value of the warrant in excess of the proceeds is considered a deemed dividend and reflected as an equity transaction in the financial statements. The Company recorded $6.0 million to interest expense related to the debt discount associated with the convertible debt during the quarter ended March 31, 2013.

On March 7, 2013, the Company closed a private placement of a new series of convertible preferred stock that resulted in the 2013 recapitalization. In this private placement, the Company issued and sold an aggregate of 4,497,035 shares of its Series Four senior preferred stock (Series Four) for an aggregate purchase price of approximately $54.0 million. Including the $6.0 million raised with the bridge financing, total gross proceeds raised during the quarter ended March 31, 2013 was approximately $60.0 million. In addition, the Company issued an aggregate of 502,919 shares of Series Four upon the share settlement of the convertible promissory notes described above that were issued in January and February 2013.

In connection with this private placement, the Company effected a one-for-120 reverse stock split of its common stock and an exchange of outstanding shares of Series One, Series Two and Series Three convertible preferred stock (Series Three) into an aggregate of 6,700,487 shares of a new series of Series Five junior preferred stock (Series Five). In addition, the Company issued an aggregate of 2,527,675 shares of Series One and Series Two upon the exercise of the warrants issued in connection with the bridge loan that were immediately exchanged for 2,095,515 shares of Series Five during the 2013 recapitalization.

The Company accounted for the 2013 recapitalization as an extinguishment of its Series One, Series Two and Series Three convertible preferred stock and recorded the Series Five shares at their fair value as of the recapitalization date. In accordance with authoritative accounting guidance, the Company recorded a gain attributable to the common stockholders on the extinguishment of the Series One, Series Two and Series Three. The gain of approximately $3.4 million represents the excess of the Series One, Series Two and Series Three over the fair value of the shares Series Five issued in connection with the recapitalization.

Initial Public Offering

In June 2013, the Company closed the initial public offering of its common stock pursuant to a registration statement on Form S-1, as amended. The Company issued and sold an aggregate of 9,627,800 shares of common stock under the registration statement at a public offering price of $15.00 per share, including 1,255,800 shares pursuant to the exercise by the underwriters of an over-allotment option. The Company received net proceeds from the initial public offering of approximately $131.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

Upon closing the initial public offering, all outstanding shares of the Series Four and Series Five were converted into 14,170,956 shares of common stock.

Follow-On Offering

In February 2014, the Company closed a follow-on public offering of its common stock pursuant to a registration statement on Form S-1, as amended. The Company issued and sold an aggregate of 5,163,265 shares of common stock under the registration statement at a public offering price of $24.50 per share, including 673,469 shares pursuant to the exercise by the underwriters of an over-allotment option. The Company received net proceeds from the follow-on public offering of approximately $118.4 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

Warrants

All of the Company's outstanding warrants were classified as liabilities as of June 30, 2014 and December 31, 2013 because they contained non-standard antidilution provisions.

The following is a summary of the Company's outstanding warrants as of June 30, 2014:

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

The following is a summary of the Company's outstanding warrants as of December 31, 2013:

	Warrant shares		Exercise price	Expiration
Common stock	1,428	$	128.00	2014
Common stock	6,250	$	128.00	2017
Common stock	7,030	$	128.00	2019 and 2020
Common stock	452	$	2,520.00	2014

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In connection with the 2013 recapitalization, all of the Series Two outstanding warrants became warrants to purchase Series Five. In connection with the Company's initial public offering all of the Series Five outstanding warrants became warrants to purchase common stock.

7. Net loss per share

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

The following tables set forth the computation of basic and diluted net income (loss) per share for common stockholders:

	Three months ended June 30,	
	2014	2013
Numerator		
Net loss attributable to common stockholders	$ (25,104)	$ (14,586)
Denominator		
Denominator for basic and diluted net loss per share	29,332,227	2,648,832
Net loss per share:		
Basic and diluted	$ (0.86)*	$ (5.51)*

* In the three months ended June 30, 2014 and 2013, the Company experienced a net loss and therefore did not report any dilutive share impact.

	Six months ended June 30,	
	2014	2013
Numerator		
Net loss	$ (39,202)	$ (29,271)
Deemed dividend	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	3,391
Net loss attributable to common stockholders	$ (39,202)	$ (44,129)
Denominator		
Denominator for basic and diluted net loss per share	27,976,847	1,326,679
Net loss per share:		
Basic and diluted	$ (1.40)*	$ (33.26)*

* In the six months ended June 30, 2014 and 2013, the Company experienced a net loss and therefore did not report any dilutive share impact.

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

	As of June 30,	
	2014	2013
Stock Options	3,182,963	2,048,737
Unvested restricted stock	729,320	1,128,672
Total	3,912,283	3,177,409

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

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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 Stock 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) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan 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.

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, 2013	2,095,592	$ 20.24		
Granted	1,174,946	$ 27.78		
Exercised	(2,750)	$ 10.85		
Forfeited	(84,825)	$ 11.61		
Outstanding at June 30, 2014	3,182,963	$ 23.26	9.09 years	$ 29,263
Vested or Expected to vest at June 30, 2014	2,956,469	$ 21.69	9.14 years	$ 27,526
Exercisable at June 30, 2014	629,159	$ 39.44	8.53 years	$ 9,018

From January 1, 2014 through June 30, 2014, the Company issued a total of 1,174,946 stock options to various employees. Of those, 183,750 were inducement grants for non-statutory stock options. The awards were made pursuant to the NASDAQ inducement grant exception as a component of our new hires' employment compensation.

The fair value of grants made in the period ended June 30, 2014 was contemporaneously estimated on the date of grant using the following assumptions:

	Six months ended June 30, 2014
Risk-free interest rate	0.11%—2.03%
Expected volatility	89%—91%
Expected term	5.5 years—6.25 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the six month period ended June 30, 2014 was $20.75 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 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:

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	Restricted Stock	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2014	1,110,226	$ 10.68
Granted	—	
Vested	(371,280)	$ 10.60
Forfeited	(9,626)	$ 10.66
Unvested at June 30, 2014	729,320	$ 10.72

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Research and development	$ 2,209	$ 1,107	$ 4,153	$ 1,364
General and administrative	2,069	774	3,830	1,138
Total	$ 4,278	$ 1,881	$ 7,983	$ 2,502

As of June 30, 2014 there was approximately $36.1 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 1998, 2009 and 2013 Plans. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.67 years.

9. Collaboration Revenue

On January 22, 2014, the Company announced the initiation of a Phase 1 clinical program in its spinal muscular atrophy collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc. (Roche) and the Spinal Muscular Atrophy Foundation which triggered a $7.5 million milestone payment from Roche. The Company considered this milestone event substantive because the applicable criteria of its revenue recognition policy would be satisfied and recorded it as collaboration revenue for the three months ended March 31, 2014.

10. Subsequent Events

On August 4, 2014, the Company was notified that the European Commission, or EC, granted conditional marketing authorization for Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The conditional marketing authorization allows the Company to market Translarna in the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland and Liechtenstein. The conditional marketing authorization is subject to an annual review by the EMA and the Company will seek to renew the approval on an annual basis until its obligations have been fulfilled and the approval is converted from a conditional approval into a full approval.

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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, 2013 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 6, 2014. 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.

Overview and Corporate Update

We are a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. Our internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. We have developed proprietary technologies that we apply in our drug discovery activities and in collaborations with leading biopharmaceutical companies.

Our lead candidate is ataluren, an investigational new drug in the US, for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. The brand name of ataluren is Translarna™.

Translarna™ for nmDMD

On August 4, 2014, we were notified that the European Commission, or EC, granted conditional marketing authorization for Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The conditional marketing authorization allows us to market Translarna in the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland and Liechtenstein. Our conditional marketing authorization is subject to an annual review by the European Medicines Agency, or EMA,

and we will 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.

We have begun our commercialization efforts and plan to launch Translarna in selected countries beginning in the first half of 2015, subject to completion of each country's market access process and timeline. Our strategy is to initially focus our commercial efforts in those countries in Europe which we believe represent a significant portion of the commercial opportunity. We are currently working on country-specific market access submissions for these target countries, which we expect to begin submitting during the fourth quarter of 2014. The market access process timeline varies from country to country and can take over eighteen months in certain circumstances. 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. We ultimately intend to market Translarna in all markets in the EEA where market access is possible.

In parallel, we have begun to pursue reimbursed expanded access programs for Translarna for nmDMD patients in selected territories, which we refer to as our EAP program. Our EAP program is intended to make Translarna available to patients before commercial product becomes available in those countries in accordance with local regulations. Funded named patient programs for Translarna, which form part of our EAP program, have already been authorized in Turkey and Spain. On July 9, 2014, the French National Agency for Medicines and Health Products Safety, or ANSM, granted a Temporary Authorization for Use, or ATU cohort. Under a named patient program a physician on behalf of the specific, or "named", patient requests access to Translarna, whereas, the ATU cohort allows for a broader temporary authorization for use for nmDMD meeting the inclusion criteria. We recently initiated the supply of Translarna for the first patients authorized under our EAP program.

In addition, we expect to seek regulatory approval for Translarna in those territories outside of Europe that will reference the European conditional marketing authorization as the basis for a local market authorization process. This will include specific countries where we have elected to market Translarna through a third-party distributor/marketing partner.

We have initiated a confirmatory Phase 3 clinical trial of Translarna for the treatment of nmDMD. We refer to this trial as the Ataluren Confirmatory Trial in DMD, or ACT DMD. We dosed the first patient in this trial in April 2013. Based on our current estimates regarding patient enrollment, we expect to complete enrollment for this trial in the near term and have initial, top-line data available in the second half of 2015. As part of the conditional marketing authorization granted by the European Commission, we are required to complete ACT DMD and submit additional efficacy and safety data from the trial. We are engaging in further dialogue with the U.S. Food and Drug Administration, or FDA, to discuss potential pathways to accelerate bringing Translarna to US patients. We estimate that a nonsense mutation is the cause of Duchenne muscular dystrophy in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union. 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 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.

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Translarna™ for nmCF

At the end of the second quarter of 2014, we initiated 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 the Ataluren Confirmatory Trial in Cystic Fibrosis, or ACT CF. ACT CF is an international, randomized, double-blind, placebo-controlled, study of Translarna in patients six years of age or older with nmCF not receiving chronic inhaled aminoglycosides. Based on our estimates regarding patient enrollment, we expect to complete enrollment for this trial in the second half of 2015 and have initial, top-line data available approximately one year later.

Translarna™ for nmMPS I and other Indications

We also plan to pursue additional indications for Translarna beyond nmDMD and nmCF and our goal is to initiate a Phase 2 proof-of-concept study in the fourth quarter of 2014 in mucopolysaccharidosis type I,or MPS I, an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of by-products of chemical reactions in the body's cells. Globally, MPS I occurs in about 1 in every 100,000 births. It is estimated that 60% to 80% of patients have their disease as a result of a nonsense mutation, which we refer to as nmMPS I. There is no cure for MPS I and enzyme replacement therapies do not sufficiently address the central nervous system, skeletal or cardiac symptoms associated with the disorder. Prognosis of patients with MPS I is poor and there is an urgent need for the development of new treatments targeting the underlying cause of MPS I.

Spinal Muscular Atrophy

We continue to advance the development of our spinal muscular atrophy, or SMA, collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. In January 2014, a Phase 1a single ascending dose, placebo-controlled clinical trial in healthy volunteers was initiated. The primary objectives of this trial were to explore safety and pharmacokinetics of the drug candidate, RG7800. This trial has now completed and a multiple dose clinical trial in SMA patients is currently in preparation. Preliminary findings in the Phase 1a study indicate that RG7800 was well-tolerated at all dose levels studied. There were no deaths, serious adverse events (SAEs) or withdrawals due to adverse events (AEs) and no dose-related trends were identified. Additionally, RG7800 had a dose-dependent effect on SMN2 splicing, as shown by a change in the ratio of full-length SMN2 mRNA to SMN2 mRNA without exon 7 (SMND7), which may be interpreted as proof of mechanism in terms of the expected pharmacodynamic effect.

BMI1

IND-enabling preclinical studies are ongoing for our development candidate, PTC596, for the treatment of chemotherapy resistant cancers through the targeting of cancer stem cells. We plan to initiate a Phase 1 study in PTC596 for the treatment of drug-resistant tumors. PTC596 is a first-in-class, oral, potent and selective inhibitor of BMI1 protein expression. Elevated levels of BMI1 are associated with more aggressive tumors and a poor prognosis in a wide variety of cancers including glioblastoma. Reducing levels of BMI1 therefore represents a promising new therapeutic strategy to treat drug-resistant cancers.

Other

We are also pursuing additional programs to expand our pipeline that are currently either at the preclinical development or discovery stage. These are focused on new treatments for multiple therapeutic areas, including neuromuscular disease, oncology and infectious diseases. In particular, we expect to declare a development candidate in our antibacterial program later this year. This program is based on a novel chemical scaffold and has the potential to address the significant need for new treatment options to combat drug resistant gonorrhea.

Funding

To date, we have financed our operations primarily through our public offering of common stock in February 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 June 30, 2014, we had an accumulated deficit of $368.0 million. We had a net loss of $39.2 million for the six months ended June 30, 2014 and a net loss of $29.3 million for the six months ended June 30, 2013.

We anticipate that our expenses will increase substantially in connection with the expansion of our commercial infrastructure as we seek to establish an international presence, particularly throughout Europe, and our efforts to 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 clinical trials for Translarna for the treatment of nmDMD and nmCF as well as our planned Phase 2 proof-of-concept study for nmMPS I. We also expect to incur ongoing research

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and development expenses for our other product candidates. In addition, we may seek marketing approval for Translarna for other indications or in other territories, which would 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. 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

Revenues

To date, we have not generated product sale revenues. We have begun our commercialization efforts for Translarna for nmDMD and we plan to launch in selected countries during the first half of 2015, subject to completion of each country's market access process and timeline. Our revenues to date have consisted of collaborative agreements revenues and grant revenues.

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

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

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. Roche is responsible for pursuing clinical development of compounds from the program, consistent with a governance structure that includes representation from us and the SMA Foundation, and then commercialization of these compounds.

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

Research and development expense

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

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;
- employee-related expenses, which include salaries and benefits, including 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 clinical trials of Translarna for the treatment of nmDMD and nmCF, commence our Phase 2 proof-of-concept study in nmMPS I, continue our research activities in our preclinical programs and initiate clinical development of other product

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candidates. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs and product candidate manufacturing costs.

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

	Three months ended June 30,		Six months ended June 30,	
	2014	2013	2014	2013
	(in thousands)		(in thousands)	
Translarna	$ 12,384	$ 10,435	$ 22,448	$ 16,790
Antibacterial	1,770	1,488	3,824	2,692
BMI1	462	1,948	1,105	1,877
Spinal muscular atrophy	812	783	1,477	1,353
Other research and preclinical	2,885	58	5,348	3,257
Total research and development	$ 18,313	$ 14,712	$ 34,202	$ 25,969

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

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

A change in the outcome of any of these variables with respect to the development of 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.

General and administrative expense

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

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resource functions. Other general and administrative expenses include facility-related costs not otherwise included in research and development expense; advertising and promotional expenses; costs associated with industry and trade shows; and professional fees for legal services, including patent-related expenses, and accounting services.

We expect that general and administrative expenses will increase in 2014 and future periods as a result of the expansion of our commercial infrastructure as we seek to establish an international presence, particularly throughout Europe, and our efforts to commercialize Translarna for the treatment of nmDMD, including significant sales and marketing, legal and regulatory, and distribution and manufacturing expenses.

Interest income, net

Interest income, net consists of interest realted to our secured debt facility and interest income earned on investments.In July 2013, we paid in full the outstanding principal and interest related to our secured debt facility.

Critical accounting policies and significant judgments and estimates

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

While our significant accounting policies are more fully described in the notes to our financial statements appearing in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013, we believe that the following accounting policies are the most critical to understanding and evaluating our financial condition and results of operations.

As an emerging growth company under the Jumpstart Our Business Startups Act of 2012, we have elected to delay the adoption of new or revised accounting standards until those standards would otherwise apply to private companies. As a result of this election, our financial statements may not be comparable to the financial statements of other public companies.

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.

To date our revenue has been generated primarily through collaborative research and development and licensing agreements and grants.

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

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

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

We evaluate all contingent consideration earned, such as a milestone payment, using the criteria as provided by the Financial Accounting Standards Board, or FASB, guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, we evaluate if milestone payments are substantive. The criteria requires that (1) we determine if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered as

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substantive milestones and will be recognized as revenue in the period that the milestone is achieved. We recognize royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

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

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

Accrued expenses

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

- fees paid to contract research organizations in connection with preclinical and toxicology studies and clinical trials;
- fees paid to investigative sites in connection with clinical trials;

· fees paid to contract manufacturers in connection with the production of clinical trial materials; and

· professional service fees.

Share-based compensation

We expect to grant additional stock options that will result in additional share-based compensation expense. 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.

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.

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

The fair value of grants made in the six months ended June 30, 2014 and 2013 was contemporaneously estimated on the date of grant using the following assumptions:

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	Six months ended June 30,	
	2014	2013
Risk-free interest rate	0.11%—2.03%	0.85%-1.69%
Expected volatility	89%—91%	87-88%
Expected term	5.50—6.25 years	5.00—6.00 years

We assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the six month period ended June 30, 2014 was $20.75 per share.

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Research and development	$ 2,209	$ 1,107	$ 4,153	$ 1,364
General and administrative	2,069	774	3,830	1,138
Total	$ 4,278	$ 1,881	$ 7,983	$ 2,502

As of June 30, 2014 there was approximately $36.1 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 1998, 2009, 2013 Plans and inducement grants made pursuant to the NASDAQ inducement grant exception as a component of our new hires’ employment compensation. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.67 years.

Results of operations

Three months ended June 30, 2014 compared to three months ended June 30, 2013

The following table summarizes revenues and selected expense and other income data for the three months ended June 30, 2014 and 2013.

	Three months ended June 30,		
(in thousands)	2014	2013	Change 2014 vs. 2013
Revenues	$ 1,677	$ 6,854	$ (5,177)
Research and development expense	18,313	14,712	3,601
General and administrative expense	8,733	6,595	2,138
Interest income (expense), net	248	(114)	362

Revenues. Revenues were $1.7 million for the three months ended June 30, 2014, a decrease of $5.2 million, or 76%, from $6.9 million for the three months ended June 30, 2013. Collaboration revenue was $1.4 million for the three months ended June 30, 2014, a decrease of $4.5 million, or 76%, from collaboration revenues of $5.9 million for the three months ended June 30, 2013. The decrease was due to a decrease in the recognition of non-cash deferred revenue compared to the same period in 2013. Grant revenue was $0.3 million for the three months ended June 30, 2014, a decrease of $0.7 million from grant revenue of $1.0 million for the three months ended June 30, 2013.

Research and development expense. Research and development expense was $18.3 million for the three months ended June 30, 2014, an increase of $3.6 million, or 24%, from $14.7 million for the three months ended June 30, 2013. The increase resulted primarily from an increase in clinical trial expenses, including the manufacturing of drug product for our clinical trials, regulatory costs associated with the efforts to obtain conditional approval for Translarna in Europe and an increase in non-cash, stock-based compensation of approximately $1.1 million.

General and administrative expense. General and administrative expense was $8.7 million for the three months ended June 30, 2014, an increase of $2.1 million, or 32%, from $6.6 million for the three months ended June 30, 2013. The increase resulted primarily from increased non-cash stock-based compensation expense of approximately $1.3 million and costs related to efforts to obtain conditional approval for Translarna in Europe, pre-commercial activities and public company costs.

Interest income (expense), net. Net interest income was $0.2 million for the three months ended June 30, 2014. The increase from interest expense of $0.1 million for the three months ended June 30, 2013 was due to interest income related to investments offset by lower interest expense resulting from the payoff of debt in July 2013.

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Six months ended June 30, 2014 compared to six months ended June 30, 2013

The following table summarizes revenues and selected expense and other income data for the six months ended June 30, 2014 and 2013.

	Six months ended June 30,		
(in thousands)	**2014**	**2013**	**Change 2014 vs. 2013**
Revenues	$ 10,894	$ 13,996	$ (3,102)
Research and development expense	34,202	25,969	8,233
General and administrative expense	16,273	11,056	5,217
Interest income (expense), net	419	(6,276)	6,695

Revenues. Revenues were $10.9 million for the six months ended June 30, 2014, a decrease of $3.1 million, or 22%, from $14.0 million for the six months ended June 30, 2013. Collaboration revenue was $10.6 million for the six months ended June 30, 2014, a decrease of $1.3 million, or 11%, from collaboration revenues of $11.9 million for the six months ended June 30, 2013. The decrease resulted primarily from a decrease in the recognition of non-cash deferred revenue compared to the same period in 2013 partially offset by the recognition of a $7.5 million milestone in our spinal muscular atrophy collaboration with Roche. Grant revenue was $0.3 million for the six months ended June 30, 2014, a decrease of $1.7 million, or 84%, from grant revenue of $2.0 million for the six months ended June 30, 2013.

Research and development expense. Research and development expense was $34.2 million for the six months ended June 30, 2014, an increase of $8.2 million, or 32%, from $26.0 million for the six months ended June 30, 2013. The increase resulted primarily from increased clinical trial expenses, including the manufacturing of drug product for our clinical trials, regulatory costs associated with the efforts to obtain conditional approval for Translarna in Europe and increase in non-cash, stock-based compensation expense of $2.8 million.

General and administrative expense. General and administrative expense was $16.3 million for the six months ended June 30, 2014, an increase $5.2 million or 47% from $11.1 million for the six months ended June 30, 2013. The increase resulted primarily from increased non-cash stock-based compensation expense of $2.7 million, costs related to efforts to obtain conditional approval for Translarna in Europe, and pre-commercial activities.

Interest expense. Interest income was $0.4 million for the six months ended June 30, 2014, an increase of $6.7 million from interest expense of $6.3 million for the six months ended June 30, 2013. The increase was due to interest income related to investments combined with lower interest expense resulting from the payoff of debt in July 2013. Interest expense for the six months ended June 30, 2013 was due to interest related to the debt discount associated with the convertible debt issued in 2013.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses. To date, we have not generated product sale revenues. We have begun our commercialization efforts for Translarna for nmDMD and we plan to launch commercially in selected countries in Europe during the first half of 2015, subject to completion of each country's market access process and timeline. We have financed our operations primarily through the issuance and sale of our common stock in our public offering in February 2014 and our initial public offering 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. 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 over-allotment 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.

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Cash flows

As of June 30, 2014, we had cash, cash equivalents and marketable securities of $226.9 million.

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

	Six months ended

(in thousands)	June 30, 2014	2013
Cash provided by (used in):		
Operating activities	$ (32,245)	$ (27,282)
Investing activities	(80,361)	(11,680)
Financing activities	118,364	190,331

Net cash used in operating activities was $32.2 million for the six months ended June 30, 2014 and $27.3 million for the six months ended June 30, 2013. The change in net cash used in operating activities primarily related to supporting clinical development and pre-commercial activities.

Net cash used in investing activities was $80.4 million for the six months ended June 30, 2014 and $11.7 million for the six months ended June 30, 2013. Cash used in investing activities was primarily related to net purchases of marketable securities for the six months ended June 30, 2014 and June 30, 2013.

Net cash provided by financing activities was $118.4 million for the six months ended June 30, 2014. Net cash provided by financing activities in 2014 was attributable to approximately $118.4 million in net proceeds from the public offering in February 2014. Net cash provided by financing activities was $190.3 million for the six months ended June 30, 2013. Net cash provided by financing activities in 2013 was primarily attributable to the $60.8 million in net proceeds that we received from the sale of Series Four preferred stock and the $131.7 million in net proceeds that we received from our initial public offering of common stock in June 2013.

Funding requirements

We anticipate that our expenses will increase substantially in connection with the expansion of our commercial infrastructure as we seek to establish an international presence, particularly throughout Europe, and our efforts to 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 clinical trials for Translarna for the treatment of nmDMD and nmCF as well as our planned Phase 2 proof-of-concept study for nmMPS I. We also expect to incur ongoing research and development expenses for our other product candidates. In addition, we may seek marketing approval for Translarna for other indications, including nmCF, or in other territories, which would 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 existing cash and cash equivalents, including the net proceeds from our public offerings of common stock, marketable securities and research funding that we expect to receive under our collaborations, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into mid-2016. 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 progress and results of confirmatory Phase 3 clinical trials of Translarna for nmDMD and nmCF as well as our planned Phase 2 proof-of-concept study for nmMPS I;

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- 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 connection with the conditional marketing authorization 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;
- the costs, timing and outcome of regulatory review of Translarna and our other product candidates;
- 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.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates and marketing, distribution or licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect 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, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual obligations

The following table summarizes our significant contractual obligations and commercial commitments as of June 30, 2014.

(in thousands)	Total	Less than 1 year	1-3 years	4-5 years	More than 5 years
Operating lease obligations(1)	$ 4,174	$ 938	$ 2,587	$ 649	$ —
Total fixed contractual obligations	$ 4,174	$ 938	$ 2,587	$ 649	$ —

(1) We lease office space under a noncancelable operating lease with a term that extends through February 2019.

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

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

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

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

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Item 3. Quantitative and Qualitative Disclosures About Market Risk.

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

Item 4. 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 June 30, 2014. The term “disclosure controls and procedures”, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated,

can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of June 30, 2014, 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.

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. These matters are subject to various uncertainties, and it is possible that some of these matters may be resolved unfavorably to us. However, we believe that the ultimate outcome of the matters that are currently pending will not have a material adverse impact on our business.

Item 1A. Risk Factors.

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

Risks Related to Our 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 expansion of our international commercial infrastructure, our commercial launch of Translarna™ (ataluren) in Europe, our efforts to obtain broader 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 June 30, 2014, we had an accumulated deficit of $368.0 million. To date, we have 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 have historically devoted substantially all of our efforts to research and development, including clinical trials. 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.

On August 4, 2014, we were notified that the European Commission granted conditional 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 conditional marketing authorization allows us to market Translarna in the European Economic Area, which is comprised of the 28 countries in the European Union as well as Norway, Iceland and Liechtenstein.

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We anticipate that our expenses will increase substantially in connection with the expansion of our commercial infrastructure as we seek to establish an international presence, particularly throughout Europe, and our efforts to 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 clinical trials for Translarna for the treatment of nmDMD and cystic fibrosis caused by nonsense mutations, or nmCF, as well as our planned Phase 2 proof-of-concept for mucopolysaccharidosis type I caused by nonsense mutations, or nmMPS I. We also expect to incur ongoing research and development expenses for our other product candidates. In addition, we may seek marketing approval for Translarna for other indications, or in other territories, which would 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.

Our ability to generate profits from operations 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:

- successfully initiating and completing confirmatory Phase 3 clinical trials of Translarna for the treatment of either or both of nmDMD and nmCF and successfully initiating clinical trials of Translarna for the treatment of additional indications, including nmMPS I;
- establishing an expanded international 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;
- successfully 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 in the countries in which we 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 conditional marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area;
- identifying patients eligible for treatment with Translarna;
- obtaining approval to market Translarna for the treatment of other indications, and expanding the territories in which we are approved to market Translarna for the treatment of nmDMD;
- 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 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.

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

We expect 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 could further increase in the event that we were to expand the geographic area covered by our commercial launch or 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 confirmatory Phase 3 clinical trials of Translarna for the treatment of nmDMD and nmCF, commence our Phase 2 proof-of-concept study in nmMPS I, and continue our research activities in our preclinical and early clinical stage programs and initiate clinical development of other product candidates. Furthermore, since the closing of our initial public offering in June 2013, we have begun to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or our commercialization efforts.

We believe that our existing cash and cash equivalents, including the net proceeds from our public offerings of common stock, marketable securities and research funding that we expect to receive under our collaborations, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into mid-2016. 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 progress and results of confirmatory Phase 3 clinical trials of Translarna for nmDMD and nmCF as well as our planned Phase 2 proof-of-concept study for nmMPS I;
- 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 connection with the conditional marketing authorization 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;
- the costs, timing and outcome of regulatory review of Translarna and our other product candidates;
- 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 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 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.

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To date, we have not generated product sale revenues. We have begun our commercialization efforts for Translarna for nmDMD and we plan to launch in selected countries during the first half of 2015, subject to completion of pricing and reimbursement negotiations. We do not currently anticipate generating significant commercial revenue from Translarna for the treatment of nmDMD during fiscal 2014. We expect that our commercial revenue, if any, generated in the next several years will be derived exclusively from sales of Translarna for the treatment of nmDMD and that commercial sales will generally be limited to countries in the European Economic Area or other territories in which we have obtained marketing authorization. Other commercial revenue, if any, will be derived from sales of products that we are not planning to have commercially available for several years, if at all.

Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or 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.

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.

Our operations to date have been limited to organizing and staffing our company, developing and securing our technology, raising capital and undertaking preclinical studies and clinical trials of our product candidates. We have not yet demonstrated our ability to successfully complete development of any product candidates, obtain marketing approvals (other than with respect to the conditional marketing authorization granted by the European Commission in August 2014 for Translarna for the treatment of nmDMD), manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions 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. We will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Risks related to the development and commercialization of our product candidates

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

We have invested a significant portion of our efforts and financial resources in the development of Translarna for nmDMD and nmCF and have recently announced our plans to initiate a Phase 2 proof-of-concept study in nmMPS I. Our ability to generate product revenues will depend heavily on the successful development and commercialization of Translarna. The success of Translarna will depend on a number of factors, including the following:

- successful completion of confirmatory Phase 3 clinical trials of Translarna in nmDMD and nmCF and the successful initiation of our Phase 2 proof-of-concept study in nmMPS I;

- the establishment of an expanded international commercial infrastructure capable of supporting product sales, marketing and distribution of Translarna;

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- implementing marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization

- the continued maintenance of conditional marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area;
- whether and when we obtain marketing approval of Translarna in additional territories and for additional or expanded indications;
- successful negotiation of favorable pricing and reimbursement 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 commercial manufacturing arrangements with third-party manufacturers;
- 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 successfully commercialize Translarna, which would materially harm our business.

The conditional marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD is conditional and 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 conditional marketing authorization.

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. 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 the conditional 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 conditional marketing authorization granted by the European Commission is subject to an annual reassessment of the risk-benefit balance by the EMA. If we fail to meet the approval conditions established for Translarna, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the EMA could vary, suspend or withdraw the marketing authorization for Translarna. This would negatively impact our anticipated revenue from Translarna and would have a material adverse effect on our business, financial condition, results of operations and growth prospects.

If clinical trials of our 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

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outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

For example, we did not achieve the primary efficacy endpoint with the pre-specified level of statistical significance in a Phase 2b clinical trial of Translarna for the treatment of nmDMD that we completed in 2010 or in a Phase 3 clinical trial of Translarna for the treatment of nmCF that we completed in 2011. Although we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that Translarna was active and showed clinically meaningful improvements over placebo in these trials, we may similarly fail to achieve the primary efficacy endpoint in ACT DMD and ACT CF, our confirmatory Phase 3 clinical trials of Translarna for these indications. If the results of our confirmatory Phase 3 clinical trials are not favorable, we may need to conduct additional clinical trials at significant cost or altogether abandon development of Translarna for either or both of nmDMD and nmCF. We also did not achieve the primary objective in one of four prior Phase 2 clinical trials that we conducted for Translarna for the treatment of nmCF in which we measured change in chloride conductance in nasal cells over the course of treatment.

Further, as part of the conditional marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD, we are required to complete ACT DMD. The conditional marketing authorization is subject to an annual reassessment of the risk-benefit balance by the EMA. If we fail to meet the approval conditions established for Translarna, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the

EMA could vary, suspend or withdraw the marketing authorization for Translarna. This would negatively impact our anticipated revenue from Translarna and would have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates could be delayed or prevented.

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

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

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, 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;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators, 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 candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; or
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the trials.

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

Our conclusions regarding the activity and potential efficacy of Translarna 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 are based on retrospective analyses of the results of these trials and nominal p-values, which are generally considered less reliable indicators of efficacy than pre-specified analyses and adjusted p-values.

After determining that we did not achieve the primary efficacy endpoint with the pre-specified level of 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, such as when two active treatments are compared to placebo or when two or more subgroups are analyzed. Nominal p-values cannot be compared to the benchmark p-value of 0.05 to determine statistical significance without being adjusted for the testing of multiple dose groups or analyses of subgroups.

Because of these limitations, regulatory authorities typically give greater weight to results from pre-specified analyses and adjusted p-values and less weight to results from post-hoc, retrospective analyses and nominal p-values. This diminishes the likelihood that the EMA will grant conditional approval of Translarna for nmCF and, even if we successfully complete our confirmatory Phase 3 clinical trials, could negatively impact the evaluation by the EMA or the FDA of our anticipated applications for full marketing approval for Translarna for the applicable indication.

Our confirmatory Phase 3 clinical trials of Translarna for nmDMD and nmCF, even if successfully completed, may not be sufficient for approval of Translarna for the applicable indication.

It is possible that the EMA or the FDA may not consider the results of our confirmatory Phase 3 clinical trials of Translarna for nmDMD or nmCF, once completed and even if successful, 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 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 product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is increased risk that the outcome of our clinical trials will not be favorable.

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

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

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

We are faced with similar challenges in connection with the design of our Phase 2 proof-of-concept study of Translarna in nmMPS I because there is also limited historical clinical trial experience for the development of drugs to treat this disorder. While clinical trials of enzyme replacement therapies conducted by third parties 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 clinical trials of Translarna in nmDMD and nmCF or our Phase 2 proof-of-concept study of Translarna in nmMPS I, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. For example, each of nmDMD, nmCF and nmMPS I 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:

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

Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of the company to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients in our confirmatory Phase 3 clinical trials of Translarna, our Phase 2 proof-of-concept study of Translarna in nmMPS I or any of our other clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

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

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

For example, although we did not observe a pattern of liver enzyme elevations in our Phase 2 or Phase 3 clinical trials of 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.

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

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

Our scientific approach focuses on the discovery and development of product candidates that target post-transcriptional control processes. While a number of commonly used drugs and a growing body of research validate the importance of post-transcriptional control processes in the origin and progression of a number of diseases, no existing drugs have been specifically designed to alter post-transcriptional control processes in the same manner as 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 conditional marketing authorization by the European Commission for Translarna for the treatment of nmDMD, we may not be successful in developing and receiving full regulatory

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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 or conditional 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 has received conditional marketing authorization for the treatment of nmDMD, Translarna and any of our other product candidates that may receive marketing or conditional 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 candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- the prevalence and severity of any side effects;
- the ability to offer our product candidates for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement; and
- any restrictions on concomitant use of other medications, such as a restriction that nmCF patients taking 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 or any of our other product candidates that receive marketing approval.

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

We have no experience in the sale or marketing of pharmaceutical products, and we may be unable to successfully commercialize Translarna. To achieve commercial success for any approved product, we must either develop our sales and marketing organization or outsource these functions to third parties. We are in the process of establishing our sales and marketing infrastructure and plan to promote Translarna in the European Economic Area for the treatment of nmDMD using both internal and external sales forces. We plan to develop our sales force in other geographic regions and for additional indications for Translarna or other product candidates, if and when such drugs are approved in the applicable region. There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of Translarna or any other product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

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

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to implement third party marketing and distribution relationships in territories where we do not pursue direct commercialization;

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- the inability of sales personnel 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 sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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.

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

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

Currently available treatments for Duchenne muscular dystrophy are only palliative. Although there are currently no marketed therapeutics approved to treat the underlying cause of nmDMD, there are other biopharmaceutical companies, including Prosensa Therapeutics and Sarepta Therapeutics, that are developing treatments for Duchenne muscular dystrophy based on a different scientific approach known as exon-skipping. Summit Corporation also has a product candidate in early clinical development designed to increase the production of the protein utrophin, which is functionally similar to dystrophin, to treat Duchenne muscular dystrophy. Nobelpharma, a Japanese company, is currently sponsoring a Phase 2 clinical trial in Japan of its product candidate NPC-14 (arbekacin sulfate), which is a generically available aminoglycoside antibiotic, in boys with nmDMD. We believe that Translarna is the only product candidate in clinical trials that is specifically designed to treat the underlying cause of nmDMD by restoring dystrophin activity.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products to manage the symptoms and side effects of cystic fibrosis. These products include Chiron Corporation's TOBI and Genentech, Inc.'s Pulmozyme. Although there are currently no marketed products approved to treat the underlying cause of nmCF, Vertex Pharmaceuticals' CFTR potentiator drug Kalydeco is approved by the FDA as a treatment for cystic fibrosis in patients six years of age and older who have a type of mutation in the CFTR gene known as a gating mutation. Vertex Pharmaceuticals also is developing two other product candidates for the treatment of cystic fibrosis in patients who have a type of mutation in the CFTR gene known as a process block mutation. We believe that 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. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or non-competitive. Our competitors may also obtain marketing approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

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

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and

retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs.

Even if we are able to commercialize Translarna 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 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. 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 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 E.U. and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for 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.

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

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

- reduced resources of our management to pursue our business strategy;
- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;

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- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- increased insurance costs, or an ability 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 clinical trials up to a $5.0 million annual aggregate limit and subject to a per claim deductible. The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage as we begin commercializing Translarna or as and when we begin commercializing any other product candidate that receives marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

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

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

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

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

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

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

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 conditional marketing authorization to treat nmDMD under a restricted label in the European Economic Area. We may not be able to maintain our conditional marketing authorization for nmDMD 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.

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

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

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers or distributors may not be able to comply with current good manufacturing practice, or cGMP, or good distribution practice, or GDP, or regulations or similar regulatory requirements outside Europe 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 products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

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

If the third parties that we engage to manufacture product for our commercial sales, preclinical tests and clinical trials should 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 our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

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

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

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

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, 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, can also lead regulatory authorities to refuse to take into account clinical trial data submitted as part of marketing authorization application, or MAA.

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 pending MAA for conditional 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 relate to waivers we granted to admit patients to our Phase 2b clinical trial of Translarna for the treatment of nmDMD in advance of formal approval of protocol amendments that would have established their eligibility for the trial, as well as our oversight of our trial sites and the completeness or sufficiency of clinical trial documentation. In response to these findings, we described to the EMA the enhanced internal procedures and controls we have implemented, and the internal quality assurance department we have established, since the conclusion of our Phase 2b clinical trial of Translarna for the treatment of nmDMD. In addition, we proposed corrective action plans to address the inspectors' specific findings. If we do not meet our commitment to the corrective actions we proposed to the EMA, we may face additional consequences, including rejection of data or other direct action by national regulatory authorities, which could require us to conduct additional clinical trials or other supportive studies to maintain our conditional marketing authorization granted by the European Commission 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 approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

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

For each of our product candidates, we plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaborations with 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 in certain countries and, as we prepare to commercialize Translarna, we anticipate that we may 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 abilities to successfully perform the functions assigned to them in these arrangements. In particular, the successful development of a product candidate from our spinal muscular atrophy program will initially depend on the success of our

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collaborations with the SMA Foundation and Roche, including whether Roche pursues clinical development of any compounds identified under the collaborations.

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

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

- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;
- we may grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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

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

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

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

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

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

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

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

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

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

and U.S. government funding must be disclosed in any resulting patent applications. Furthermore, our rights in such inventions are subject to government license rights and certain restrictions on manufacturing products outside the United States.

Risks Related to our Intellectual Property

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

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States and in certain foreign jurisdictions related to our novel technologies and product candidates that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research

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and development output before it is too late to obtain patent protection. Moreover, if we license technology or product candidates from third parties in the future, these license agreements may not permit us to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering this intellectual property. These agreements could also give our licensors the right to enforce the licensed patents without our involvement, or to decide not to enforce the patents at all. Therefore, in these circumstances, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

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

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

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

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

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

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

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file claims, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the

patent's claims narrowly or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question.

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, inter partes review or post-grant review proceedings before the U.S. Patent and Trademark Office. The risks of being involved in such litigation and proceedings may also increase as our product candidates approach commercialization, and as we gain greater visibility as a public company. Third parties may assert infringement claims against us based on existing or future intellectual property rights. We may not be aware of all such intellectual property rights potentially relating to our product candidates. For example, we have not conducted a recent freedom-to-operate search or analysis for Translarna. 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 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 patent applications specifically discloses Translarna. In order to successfully challenge the validity of any issued U.S. patent, we would need to overcome a presumption of validity. This burden is a high one requiring us to present clear and convincing evidence as to the invalidity of these claims. There is no assurance that a court would find these claims to be invalid. In addition, we believe that our testing of 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 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.

Any trademark we intend to use for our product candidates, including Translarna™, will require that we seek trademark registration worldwide. Trademark registration is a territory-specific and we must apply for registration in the US as well as any other country where we intend to commercialize product. Failure to obtain the appropriate registrations may place our use of the trademark at risk or make if 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 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 candidates.

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Risks Related to Regulatory Approval of our Product Candidates

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

Our product candidates, including Translarna, 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 have received conditional marketing authorization to market Translarna in the European Economic Area, but have not otherwise received marketing approval for Translarna or any of our other product candidates from regulatory authorities in any jurisdiction. In 2011, we submitted a new drug application, or NDA, to the FDA for approval of Translarna for the treatment of nmDMD. The FDA refused to file this NDA on the grounds that the NDA did not contain substantial evidence of effectiveness based on the single placebo controlled Phase 2b clinical trial conducted to date.

We have only limited experience in filing and supporting the applications necessary to obtain marketing approvals for product candidates and expect to rely on third-party contract research organizations to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate’s safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that 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.

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

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

In the European Union, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. For a drug such as 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 our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product. In addition, orphan drug exclusivity will not prevent the approval of a product that is the same drug as our product candidate 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.

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

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. We have obtained a fast track designation from the FDA for 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.

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

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

- restrictions on such products, manufacturers or manufacturing processes;
- changes to or restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to implement a REMS;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;

- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions; or

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- the imposition of civil or criminal penalties.

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

Failure to obtain or maintain regulatory approval, including price and reimbursement approval, in international jurisdictions would prevent us from marketing our products abroad.

In order to market and sell Translarna and our other products in the European Union and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. 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.

In addition, some countries outside the United States, including in the European Economic Area, require approval of the sales price of a drug before it can be marketed. In many countries, separate procedures must be followed to obtain reimbursement. We may not obtain additional marketing, pricing or reimbursement approvals outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for additional marketing approvals and may not receive necessary approvals to further commercialize our products in any market. Regulatory approvals in countries outside the United States do not ensure pricing approvals in those countries or in any other countries, and regulatory approvals and pricing approvals do not ensure that reimbursement will be obtained

For example, each country in the European Economic Area 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 will not be able to commence commercial sales of Translarna for the treatment of nmDMD pursuant to the conditional marketing authorization granted by the European Commission in any particular member state of the European Economic Area until we conclude the applicable pricing and reimbursement negotiations and comply with any licensing, employment or related regulatory requirements in that country.

We are in the process of preparing pricing and reimbursement submissions with respect to Translarna for the treatment of nmDMD in those European countries where pricing and reimbursement approvals are required for launch. This process varies from country to country and can take over 18 months to complete. We cannot predict the timing of Translarna's launch in countries where we are awaiting pricing and reimbursement guidelines. If we experience delays and unforeseen difficulties in obtaining pricing and reimbursement approvals, planned launches in the affected countries would be delayed and our anticipated revenue from Translarna and our growth prospects could be negatively affected. 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. We may not be able to file for additional marketing approvals and may not receive all necessary approvals to commercialize our products in any market.

Our ability to obtain and maintain conditional marketing authorizations in the European Union is limited to specific circumstances and subject to several conditions and obligations. Conditional marketing authorizations based on incomplete clinical data may be granted for a limited number of listed medicinal products for human use, including products designated as orphan medicinal products under E.U. law, if (1) the risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) unmet medical needs will be fulfilled and (4) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations, including with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data, may be specified in the conditional marketing authorization.

Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions. The conditional marketing authorization for Translarna for the treatment of nmDMD granted by the European Commission is conditioned on our ability to complete ACT DMD. If the results of ACT DMD are not favorable, the European Commission may decline to renew our conditional marketing authorization or require additional clinical trials. Likewise, even if we obtain conditional approval for Translarna for the treatment of nmCF, we may not be able to renew such conditional approval. A failure to renew any conditional approval that has been granted or that we may obtain prior to full approval for the applicable indication would prevent us from continuing to market Translarna for such indication.

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Our initial commercial launch of Translarna is planned to take place in countries that tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the member states of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various E.U. member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidate to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be adversely affected.

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

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

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 under applicable federal and state healthcare laws and regulations, and equivalent laws and regulations in Europe, 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-US 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.

- 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. statute has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse, and healthcare laws and regulations.

- 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 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 U.S. 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.

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- 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. For example, the off-label promotion of medicinal products is prohibited in the European Union. The applicable laws at European Union level and in the individual European Union 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.

- Statutory requirements to disclose publicly payments made to physicians, including in certain European Union member states and the U.S. 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.

- Laws governing the advertising and promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. For example, legislation adopted by individual EU member states that may apply to the advertising and promotion of medicinal products require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, as approved by the competent authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion.

- 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 EU. The provision of benefits or advantages to physicians is also governed by the national laws of the EU member states. 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 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

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

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

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

Risks Related to Employee Matters and Managing Growth

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

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

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

We 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 anticipated commercial launch of Translarna, we expect to experience significant growth in our employee base for sales, marketing, operational, managerial, financial, human resources, drug development, regulatory 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

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substantial amount of time to managing these growth activities. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to our Common Stock

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

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

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

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

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:

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

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

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until December 31, 2018, provided that, if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have annual gross revenues of $1 billion or more in any fiscal year, we would cease to be an emerging growth company as of December 31 of the applicable year. We also would cease to be an emerging growth company if we issue more than $1 billion of non-convertible debt over a three-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We may choose to take advantage of some, but not all, of the available exemptions. We 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, and our management is and will continue to be required to devote substantial time to new compliance initiatives.

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

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

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

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. A significant number of our shares are currently restricted as a result of securities laws, but will be able to be sold, subject to any applicable volume limitations under federal laws with respect to affiliate sales, in the near future. Moreover, certain holders 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 also have registered on a Form S-8 registration statement all shares of common stock that we may issue under our equity compensation plans. As a result, these shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. In addition, certain of our employees, executive officers and directors have 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.

Use of Proceeds from Registered Securities

On June 25, 2013, we closed our initial public offering of 9,627,800 shares of our common stock, including 1,255,800 shares of our common stock pursuant to the exercise by the underwriters of an over-allotment option, at a public offering price of $15.00 per share for an aggregate offering price of approximately $144.4 million. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-188657), which was declared effective by the SEC on June 19, 2013.

We received aggregate net proceeds from the offering of approximately $131.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. None of the underwriting discounts and commissions or other offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours.

As of June 30, 2014, we have used approximately $22.9 million of the net offering proceeds primarily to fund the clinical development of Translarna for the treatment of nmDMD and nmCF, to seek marketing approval in the European Union and the United States for Translarna for these indications, for pre-approval commercial efforts for Translarna, to fund research and development of Translarna for additional indications and for our earlier stage programs, and for working capital and other general corporate purposes. We are holding a significant portion of the balance of the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

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: August 7, 2014

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.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheet at December 31, 2013 and June 30, 2014 (unaudited), (ii) Statements of Operations for the three month period ended June 30, 2013 and 2014 and the six month period ended June 30, 2013 and 2014, (iii) Statements of Cash Flows for the six month period ended June 30, 2013 and 2014, and (iv) Notes to Financial Statements (unaudited).

In accordance with Rule 406T of Regulation S-T, the XBRL related information in Exhibit 101 to this Quarterly Report on Form 10-Q is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act, is deemed not filed for purposes of Section 18 of the Exchange Act, and otherwise is not subject to liability under these sections.

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, Ph.D., 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)) 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) 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

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

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

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

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

Date: August 7, 2014

By: /s/ Stuart W. Peltz
Stuart W. Peltz, Ph.D.
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)) 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) 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

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

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

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

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

Date: August 7, 2014

By: /s/ Shane Kovacs
Shane Kovacs
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended June 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, Ph.D., Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: August 7, 2014

By: /s/ Stuart W. Peltz
Stuart W. Peltz, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended June 30, 2014 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: August 7, 2014

By: /s/ Shane Kovacs
Shane Kovacs
Chief Financial Officer
(Principal Financial Officer)