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

FORM 10-Q

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

For the quarterly period ended September 30, 2014

OR

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

For the transition period from _____ to _____

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	93-0797222
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
215 First Street, Suite 415 Cambridge, MA (Address of principal executive offices)	**02142** (Zip Code)

Registrant's telephone number, including area code: (617) 274-4000

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

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

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller Reporting Company	☐

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock with $0.0001 par value	41,309,944
(Class)	(Outstanding as of October 31, 2014)

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share amounts)

	As of September 30, 2014	As of December 31, 2013
Assets		
Current Assets:		
Cash and cash equivalents	$ 52,299	$ 256,965
Short-term investments	183,718	—
Accounts receivable	3,362	3,530
Restricted investments	4,000	7,250
Other current assets	26,436	3,061
Total Current Assets	269,815	270,806
Restricted investments	647	647
Property and equipment, net of accumulated depreciation and amortization of $18,865 and $17,328 as of September 30, 2014 and December 31, 2013, respectively	37,178	15,049
Patent costs, net of accumulated amortization of $1,972 and $1,622 as of September 30, 2014 and December 31, 2013, respectively	5,689	5,042
Other assets	5,729	25
Total Assets	$ 319,058	$ 291,569
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$ 967	$ 8,080
Accrued expenses	14,824	14,601
Current portion of long-term debt	97	92
Current portion of notes payable	2,415	—
Warrant liability	—	9,006
Deferred revenue	3,362	3,299
Other liabilities	1,061	888
Total Current Liabilities	22,726	35,966
Long-term debt	1,501	1,576
Notes payable	2,262	—
Deferred rent	6,365	6,835
Total Liabilities	32,854	44,377
Commitments and contingencies		
Stockholders' Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 50,000,000 shares authorized; 41,304,851 and 37,751,920 issued and outstanding as of September 30, 2014 and December 31, 2013, respectively	4	4
Additional paid-in capital	920,864	790,424
Accumulated other comprehensive loss	(55)	—
Accumulated deficit	(634,609)	(543,236)
Total Stockholders' Equity	286,204	247,192
Total Liabilities and Stockholders' Equity	$ 319,058	$ 291,569

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenue from research contracts and other grants	$ 1,059	$ 4,168	$ 9,730	$ 11,593
Operating expenses:				
Research and development	21,852	21,087	63,399	47,833
General and administrative	12,882	8,014	35,398	21,195
Operating loss	(33,675)	(24,933)	(89,067)	(57,435)
Other income (loss):				
Interest income and other, net	193	63	473	281
Gain (loss) on change in warrant valuation	4,256	(17,160)	(2,779)	(46,011)
Total other income (loss)	4,449	(17,097)	(2,306)	(45,730)
Net loss	$ (29,226)	$ (42,030)	$ (91,373)	$ (103,165)
Other comprehensive income (loss):				
Unrealized loss on available-for-sale securities	(21)	—	(55)	—
Total other comprehensive loss	(21)	—	(55)	—
Comprehensive loss	$ (29,247)	$ (42,030)	$ (91,428)	$ (103,165)
Net loss per share — basic and diluted	$ (0.71)	$ (1.24)	$ (2.31)	$ (3.17)
Weighted average number of common stock outstanding for computing basic and diluted net loss per share	41,066	33,943	39,595	32,588

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	For the Nine Months Ended September 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	$ (91,373)	$ (103,165)
Adjustments to reconcile net loss to net cash flows used in operating activities:		
Depreciation and amortization	2,532	998
Amortization of premium on available-for-sale securities	1,817	—
Loss on abandonment of patents and disposal of property and equipment	52	460
Stock-based compensation	14,578	7,476
Loss on change in warrant valuation	2,779	46,011
Non-cash interest expense	31	—
Changes in operating assets and liabilities, net:		
Net decrease (increase) in accounts receivable	168	(2,009)
Net (increase) decrease in other assets	(29,168)	1,489
Net (decrease) increase in accounts payable, accrued expenses and other liabilities	(4,506)	1,867
Net cash used in operating activities	(103,090)	(46,873)
Cash flows from investing activities:		
Release and maturity of restricted investments	3,250	—
Purchase of restricted investments	—	(7,807)
Purchase of property and equipment	(22,305)	(1,762)
Patent costs	(1,062)	(1,281)
Purchase of available-for-sale securities	(272,189)	—
Maturity of available-for-sale securities	86,599	—
Net cash used in investing activities	(205,707)	(10,850)
Cash flows from financing activities:		
Proceeds from exercise of options and warrants and the sale of common stock, net of offering costs	104,201	143,951
Repayments of long-term debt	(70)	(67)
Other financing activities, net	—	(178)
Net cash provided by financing activities	104,131	143,706
(Decrease) increase in cash and cash equivalents	(204,666)	85,983
Cash and cash equivalents:		
Beginning of period	256,965	187,661
End of period	$ 52,299	$ 273,644
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 60	$ 123
Supplemental schedule of non-cash investing activities and financing activities:		
Issuance of common stock in satisfaction of warrants and other liabilities	$ 11,785	$ 75,210
Tenant improvements paid by landlord	$ 154	$ 3,692
Issuance of debt in relation to the purchase of certain real and personal property located in Andover, Massachusetts	$ 4,613	$ —
Capitalized interest	$ 36	$ —
Property and equipment included in accrued expenses	$ 1,165	$ —

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries ("Sarepta" or the "Company") is a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying its proprietary platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. The Company is focused on advancing the development of its Duchenne muscular dystrophy ("DMD") drug candidates, including its lead product candidate, eteplirsen, for which the Company is in process of conducting or starting several studies. These include an ongoing open label extension study following completion of its initial Phase IIb clinical trials, several clinical trials in Exon 51 amenable genotypes, including a confirmatory study in ambulatory patients, studies on participants with early stage and advanced stage DMD and a placebo-controlled confirmatory study with one or more of the Company's follow-on DMD exon-skipping drug candidates. Additionally, the Company is working on a Phase I/IIa clinical trial for an Exon 53 skipping product candidate in the European Union ("E.U.") and has an open investigational new drug ("IND") application for its Exon 45 skipping product candidate for which it plans to begin a clinical trial early next year. The Company is also developing therapeutics for the treatment of infectious diseases. The Company was developing product candidates for Ebola and Marburg under a now expired Department of Defense ("DoD") contract and further development of these product candidates would be conditioned, in part, on obtaining additional funding, collaborations or emergency use.

The Company has not generated any revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Even if the Company does achieve revenue from product sales, it is likely to continue to incur operating losses in the near term.

As of September 30, 2014, the Company had approximately $240.7 million of cash, cash equivalents and investments, consisting of $52.3 million of cash and cash equivalents, $183.7 million of short-term investments and $4.6 million of restricted investments. The Company believes that its balance of cash, cash equivalents and investments is sufficient to fund its current operational plan for the next twelve months. The Company may pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations with or license its technology to other companies.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013.

Estimates and Uncertainties

The preparation of the unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates. Significant items subject to such estimates and assumptions include the valuation of stock-based awards and liability classified warrants, research and development expenses and revenue recognition.

Reclassification

The Company has revised the presentation as well as the caption of certain current liabilities within the unaudited condensed consolidated balance sheets to conform to the current period presentation. "Accrued liabilities" of $9.6 million as of December 31, 2013 is reclassified from "accounts payable" to "accrued liabilities". "Accrued employee compensation" of $5.0 million as of December 31, 2013 is also included within "accrued liabilities". The reclassification had no impact on total current liabilities or total liabilities.

Additionally, the Company has revised the presentation as well as the caption of certain cash flows from operating activities within the unaudited condensed consolidated statements of cash flows to conform to the current period presentation. "Net decrease in other assets" is broken out from "Net increase in accounts receivable and other assets" and presented gross on the unaudited condensed consolidated statements of cash flows. This revision had no impact on net cash used in operating activities or change in cash and cash equivalents.

2. RECENT ACCOUNTING PRONOUNCEMENTS

In August 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-15 which requires an entity's management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued. Substantial doubt about an entity's ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will not be able to meet its obligations as they become due within one year after the date that the financial statements are issued or available to be issued. If conditions or events raise substantial doubt about an entity's ability to continue as a going concern, but the substantial doubt is alleviated as a result of consideration of management's plans to mitigate those relevant conditions or events, the entity is required to disclose (1) principal conditions or events that raise substantial doubt about the entity's ability to continue as a going concern, (2) management's evaluation of the significance of those conditions or events in relation to the entity's ability to meet its obligations, and (3) management's plans that alleviate substantial doubt about the entity's ability to continue as a going concern. However, if conditions or events raise substantial doubt about an entity's ability to continue as a going concern, and substantial doubt is not alleviated after consideration of management's plans, an entity should include a statement in the footnote indicating that there is substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued. ASU No. 2014-15 is effective for the annual period ending after December 15, 2016, with early adoption permitted. The Company has not adopted this guidance as of September 30, 2014. Based on the Company's financial condition as of September 30, 2014, the Company does not expect the adoption of this guidance to have a material effect on its consolidated financial statements.

In June 2014, the FASB issued ASU No. 2014-12 which requires that companies that issue stock-based awards treat a performance target that affects vesting and that could be achieved after the requisite service period as a performance condition. ASU No. 2014-12 is effective for fiscal years after December 15, 2015, with early adoption permitted. The Company elected to adopt this ASU early but does not expect the adoption of this guidance to have a material effect on its consolidated financial statements as the performance targets of the Company's stock-based awards with performance conditions must be achieved prior to the end of the requisite service period.

In June 2014, the FASB issued ASU No. 2014-10, which eliminates the concept of a development stage entity ("DSE") in its entirety from U.S. GAAP. Under existing guidance, DSEs are required to report incremental information, including inception-to-date financial information, in their financial statements. A DSE is an entity devoting substantially all of its efforts to establishing a new business and for which either planned principal operations have not yet commenced or have commenced but there have been no significant revenues generated from that business. Entities classified as DSEs will no longer be subject to these incremental reporting requirements after adopting ASU No. 2014-10. ASU No. 2014-10 is effective for fiscal years beginning after December 15, 2014, with early adoption permitted. Retrospective application is required for the elimination of incremental DSE disclosures. Prior to the issuance of ASU No. 2014-10, the Company had met the definition of a DSE since its inception. The Company elected to adopt this ASU early and, therefore, eliminated the incremental disclosures previously required of DSEs.

In May 2014, the FASB issued ASU No. 2014-09, which amends the guidance for accounting for revenue from contracts with customers. This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, *Revenue Recognition,* and creates a new Topic 606, *Revenue from Contracts with Customers*. Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. This guidance is effective for fiscal years beginning after December 15, 2016, with early adoption not permitted. Two adoption methods are permitted: retrospectively to all prior reporting periods presented, with certain practical expedients permitted; or retrospectively with the cumulative effect of initially adopting the ASU recognized at the date of initial application. The Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements.

3. ACCOUNTS RECEIVABLE

The Company's accounts receivable primarily arise from government research contracts and other grants. They are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily from government agencies and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. As of

September 30, 2014 and December 31, 2013, the accounts receivable balance included unbilled receivables of $2.5 million and $2.4 million, respectively. Approximately $2.4 million of the unbilled receivables as of September 30, 2014 are subject to government audit and will not be collected until the completion of the audit.

4. FAIR VALUE MEASUREMENTS

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements:

- Level 1 — quoted prices for identical instruments in active markets;
- Level 2 — quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3 — valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value and indicate the level within the fair value hierarchy of the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of September 30, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 576	$ 576	$	$ —
Commercial paper	2,995	—	2,995	
Government and government agency bonds	92,822	—	92,822	—
Corporate bonds	87,901	—	87,901	—
Certificates of deposit	4,647	4,647	—	—
Total assets	$ 188,941	$ 5,223	$ 183,718	$ —

	Fair Value Measurement as of December 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 185,000	$ 185,000	$ —	$ —
Certificates of deposit	7,897	7,897	—	—
Total assets	$ 192,897	$ 192,897	$ —	$ —

	Fair Value Measurement as of September 30, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants	$ —	$ —	$ —	$ —
Total liabilities	$ —	$ —	$ —	$ —

	Fair Value Measurement as of December 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants	$ 9,006	$ —	$ —	$ 9,006
Total liabilities	$ 9,006	$ —	$ —	$ 9,006

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of September 30, 2014.

The Company's assets with fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds and corporate bonds. These assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing market observable data.

The Company's liabilities with fair value categorized as Level 3 within the fair value hierarchy consist of warrants issued in January and August 2009. The fair value of these liabilities is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs in the model. As of September 30, 2014, all outstanding warrants issued in January and August 2009 had been exercised or expired. For additional information related to the determination of fair value of warrants and a reconciliation of changes in fair value, please read *Note 7, Warrants* of the unaudited condensed consolidated financial statements.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximate fair value because of the immediate or short-term maturity of these financial instruments. The carrying amounts reported for long-term debt and notes payable approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated:

	As of September 30, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 52,299	$ —	$ —	$ 52,299
Commercial paper	2,995	—	—	2,995
Government and government agency bonds	92,846	1	(25)	92,822
Corporate bonds	87,932	—	(31)	87,901
Total	$236,072	$ 1	$ (56)	$236,017
As reported:				
Cash and cash equivalents	52,299	—	—	52,299
Short-term investments	183,773	1	(56)	183,718
Total	$236,072	$ 1	$ (56)	$236,017

	As of December 31, 2013			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$256,965	$ —	$ —	$256,965
Total	$256,965	$ —	$ —	$256,965
As reported:				
Cash and cash equivalents	$256,965	$ —	$ —	$256,965
Total	$256,965	$ —	$ —	$256,965

6. ACQUISITION OF ASSETS

On May 22, 2014, the Company entered into a Purchase and Sales Agreement with Eisai, Inc. to acquire certain real and personal property located in Andover, Massachusetts. The aggregate purchase price, including certain fees and taxes was approximately $15.1 million, of which approximately $10.1 million was paid at closing and the remaining $5.0 million will be paid in two installments by July 15, 2015 and January 15, 2016. On July 15, 2014, the closing of the purchase of the real and personal property was completed. In connection with this transaction, the Company recorded $14.8 million as property and equipment, $2.4 million as current portion of notes payable and $2.3 million as notes payable on the unaudited condensed consolidated balance sheets as of September 30, 2014.

7. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in January and August 2009 were classified as liabilities as opposed to equity due to their settlement terms which required settlement in registered shares. The outstanding warrants classified as liabilities were recorded on the unaudited condensed consolidated balance sheets and adjusted to fair value at each financial reporting period, with changes in the fair value being recorded as "Gain (loss) on change in warrant valuation" in the unaudited condensed consolidated statements of operations and comprehensive loss. Fair value was determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs used in the model. As of September 30, 2014, there were no outstanding warrants as all warrants issued in January and August 2009 have been exercised or expired.

The following table summarizes the reconciliation of the change in value of the Company's liability classified warrants for each of the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Balance at beginning of the period	$ 9,826	$ 79,116	$ 9,006	$ 65,193
(Decrease) increase in value of warrants	(4,256)	17,160	2,779	46,011
Reclassification to stockholders' equity upon exercise of warrants	(5,570)	(60,282)	(11,785)	(75,210)
Balance at end of the period	$ —	$ 35,994	$ —	$ 35,994

The following table summarizes the Company's warrant activity for each of the periods indicated:

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2014		2013		2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Warrants outstanding at beginning of the period	501,494	$ 10.20	2,628,923	$ 8.41	791,508	$ 10.05	3,127,618	$ 8.48
Exercised	(475,901)	10.32	(1,669,640)	7.62	(765,915)	10.12	(2,168,335)	7.91
Expired	(25,593)	7.92	—	—	(25,593)	7.92	—	—
Warrants outstanding at end of the period	—	$ —	959,283	$ 9.78	—	$ —	959,283	$ 9.78
Warrants exercisable at end of the period	—	$ —	959,283	$ 9.78	—	$ —	959,283	$ 9.78

8. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of September 30, 2014	As of December 31, 2013
	(in thousands)	
Accrued contract manufacturing costs	$ 2,557	$ 1,414
Accrued facility-related costs	1,116	2,843
Accrued contract research costs	2,463	2,785
Accrued employee compensation costs	5,222	5,048
Accrued professional fees	2,682	1,235
Others	784	1,276
Total accrued expenses	$ 14,824	$ 14,601

9. EQUITY FINANCINGS

In April 2014, the Company sold approximately 2.7 million shares of common stock at an offering price of $38.00 per share. The Company received aggregate net proceeds of approximately $94.5 million, after deducting the underwriting discounts and offering related transaction costs.

In July 2013, the Company entered into an At-The-Market ("ATM") offering ("2013 ATM") allowing the Company to sell, at its option, up to an aggregate of $125.0 million of shares of common stock at market prices. Through September 30, 2013, the Company sold approximately 3.4 million shares under the 2013 ATM, generating $123.0 million in net proceeds and completed the sales of common stock available under the arrangement.

In January 2013, the Company sold approximately 87,000 shares of common stock through an ATM offering that originally commenced in September 2012 ("2012 ATM"). The sales in January 2013 generated $2.1 million in net proceeds and fully exhausted the sales of stock available under the 2012 ATM sales agreement.

10. GOVERNMENT CONTRACTS

The Company recognizes revenue from U.S. and E.U. government research contracts and other grants during the period in which the related expenditures are incurred and presents revenue and related expenses gross in the unaudited condensed consolidated statements of operations and comprehensive loss. In the periods presented, substantially all of the revenue generated by the Company was derived from government research contracts.

The following table summarizes the revenue for each of the Company's contracts with the U.S. and E.U. governments for each of the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
July 2010 Agreement *(Ebola and Marburg IV)*	$ 1,043	$ 2,444	$ 6,816	$ 7,134
August 2012 Agreement (*Intramuscular*)	—	514	—	2,759
European Union SKIP-NMD Agreement *(DMD)*	16	536	1,405	599
July 2013 Children's National Medical Center Agreement *(DMD)*	—	674	659	674
Carolinas Medical Center Agreement (DMD)	—	—	850	—
Other Agreements	—	—	—	427
Total	$ 1,059	$ 4,168	$ 9,730	$ 11,593

July 2010 Agreement (Ebola and Marburg Intravenous administration)

In July 2010, the Company was awarded the DoD contract managed by its Joint Project Manager Medical Countermeasure Systems ("JPM-MCS") program for the advanced development of its hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003, against Ebola and Marburg viruses, respectively. In February 2012, the Company announced that it received permission from the FDA to proceed with a single oligomer from AVI-7288, one of the two components that make up AVI-6003, as the lead product candidate against Marburg virus infection. In August 2012, the Company received a stop-work order related to the Ebola virus portion of the contract and, in October 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The Marburg portion of the contract was structured into four segments and had an aggregate remaining period of performance spanning approximately four years if the DoD exercised its options for all segments. Activities under the first segment began in July 2010 and included preclinical studies and Phase I studies in healthy volunteers. In February 2014, the Company announced positive safety results from the Phase I multiple ascending dose study of AVI-7288. The remaining Marburg portion of the contract expired in July 2014. The majority of the revenue under this contract has been recognized as of September 30, 2014 and only revenue for contract finalization, if any, is expected in the future.

For the three months ended September 30, 2014 and 2013, the Company recognized $1.0 million and $2.4 million, respectively, as revenue under this agreement. For the nine months ended September 30, 2014 and 2013, the Company recognized $6.8 million and $7.1 million, respectively, as revenue under this agreement.

August 2012 Agreement (Intramuscular)

In August 2012, the Company was awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, the Company's candidate for treatment of the Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. Accordingly, no revenue was recognized since the conclusion of the contract. For the three and nine months ended September 30, 2013, the Company recognized $0.5 million and $2.8 million, respectively, as revenue under this agreement.

European Union SKIP-NMD Agreement (DMD)

In November 2012, the Company entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic. For the three months ended September 30, 2014 and 2013, the Company recognized less than $0.1 million and slightly more than $0.5 million, respectively, as revenue under this agreement. For the nine months ended September 30, 2014 and 2013, the Company recognized $1.4 million and $0.6 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of September 30, 2014 and only revenue for contract finalization, if any, is expected in the future.

July 2013 Children's National Medical Center ("CNMC") Agreement (DMD)

In July 2013, the Company entered into an agreement totaling $1.3 million to provide drug products to CNMC to conduct research related to one of the Company's DMD programs. For the three months ended September 30, 2014 and 2013, $0 and $0.7 million was recognized as revenue under this agreement, respectively. For each of the nine months ended September 30, 2014 and 2013, $0.7 million was recognized as revenue under this agreement. Revenue under this agreement was fully recognized as of March 31, 2014.

Carolinas Medical Center ("CMC") Agreement (DMD)

The Company entered into a collaboration agreement with CMC to co-develop one of the Company's DMD programs. Under the agreement, CMC was obligated to reimburse certain preclinical costs incurred by the Company. All preclinical work was completed and the Company recognized revenue of $0 and $0.9 million for the three and nine months ended September 30, 2014, respectively.

11. STOCK-BASED COMPENSATION

The Company's equity incentive plans allow for the granting of a variety of stock awards. To date, the Company has granted stock options, restricted stock awards, restricted stock units and stock appreciation rights. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the vesting period of the grants.

Stock Options

The Company has granted stock options with both service- and performance-based criteria. In general, stock options granted vest over four years and have a ten-year term. Through the submission of an Investigational New Drug ("IND") application in June 2014, 20% of performance awards were triggered to be eligible to vest subject to the remaining service conditions of the awards. The Company has recognized approximately $0.1 million and $0.7 million in stock-based compensation expense related to the options with performance-based criteria for the three and nine months ended September 30, 2014, respectively.

The following table summarizes the Company's stock option activity for each of the periods indicated:

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2014		2013		2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	5,228,373	$ 24.56	3,976,189	$ 21.82	4,190,367	$ 23.46	2,522,522	$ 11.76
Granted	237,050	21.98	309,226	39.17	1,540,085	26.61	2,063,396	34.86
Exercised	(4,742)	8.85	(76,465)	8.76	(79,346)	11.69	(196,012)	8.51
Canceled or expired	(331,462)	21.19	(58,116)	20.62	(521,887)	22.63	(239,072)	11.51
Grants outstanding at end of the period	5,129,219	$ 24.67	4,150,834	$ 23.37	5,129,219	$ 24.67	4,150,834	$ 23.37
Grants exercisable at end of the period	1,859,285	$ 18.61	891,157	$ 11.60	1,859,285	$ 18.61	891,157	$ 11.60

The fair values of stock options granted during the periods presented were measured on the date of grant using the Black-Scholes-Merton option-pricing model, with the following assumptions:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
Risk-free interest rate(1)	1.5 – 1.7%	0.7 – 1.4%	1.5 – 1.7%	0.7 – 1.4%
Expected dividend yield(2)	0%	0%	0%	0%
Expected lives(3)	4.7 – 4.8 years	4.9 – 5.0 years	4.7 – 4.9 years	4.9 – 5.0 years
Expected volatility(4)	96.7 – 103.0%	80.0 – 88.9%	93.0 – 103.0%	80.0 – 88.9%

(1) The risk-free interest rate is estimated using an average of Treasury bill interest rates over a historical period commensurate with the expected term of the option that correlates to the prevailing interest rates at the time of grant.
(2) The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends in the future.
(3) The expected lives are estimated using expected and historical exercise behavior.
(4) The expected volatility is estimated using a blend of calculated volatility of the Company's common stock over a historical period and implied volatility in exchange-traded options of the Company's common stock.

The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

Restricted Stock Awards ("RSA")

The Company grants RSAs to members of its board of directors. The weighted-average grant date fair value of RSAs is based on the market price of the Company's common stock on the date of grant. The fair value is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The following table summarizes the Company's RSA activity for each of the periods indicated:

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2014		2013		2014		2013	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	6,000	$ 29.03	10,998	$ 23.63	6,000	$ 34.92	4,998	$ 10.08
Granted	—	—	—	—	6,000	29.03	6,000	34.92
Vested	—	—	(4,998)	10.08	(6,000)	34.92	(4,998)	10.08
Grants outstanding at end of the period	6,000	$ 29.03	6,000	$ 34.92	6,000	$ 29.03	6,000	$ 34.92

Restricted Stock Units ("RSU")

The Company granted RSUs to employees in 2012. The weighted-average grant date fair value of RSUs is based on the market price of the Company's common stock on the date of grant. The fair value is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. All outstanding RSUs from December 31, 2013 vested during the 9-month period ended September 30, 2014. The following table summarizes the Company's RSU activity for each of the periods indicated:

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2014		2013		2014		2013	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	—	$ —	13,325	$ 5.40	6,507	$ 5.40	38,260	$ 6.32
Vested	—	—	(264)	5.40	(6,507)	5.40	(24,858)	6.81
Canceled or expired	—	—	(33)	5.40	—	—	(374)	5.40
Grants outstanding at end of the period	—	$ —	13,028	$ 5.40	—	$ —	13,028	$ 5.40

Stock Appreciation Rights ("SAR")

The Company issues SARs to employees on the same terms as the stock options granted to employees. The grant date fair value of the SARs is determined using the same valuation assumptions as for the stock options described above. Stock-based compensation expense is recognized on a straight-line basis over the vesting period of the SARs. The following table summarizes the Company's SAR activity for each of the periods indicated:

	For the Three and Nine Months Ended September 30,			
	2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	170,000	$ 18.18	170,000	$ 18.18
Grants outstanding at end of the period	170,000	$ 18.18	170,000	$ 18.18
Grants exercisable at end of the period	82,291	$ 17.75	18,958	$ 10.08

Employee Stock Purchase Plan ("ESPP")

Under the Company's ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first business day and the last business day of the relevant purchase period. The fair values of stock purchase rights are estimated using the Black-Scholes-Merton option-pricing model. The 24-month award period will end on August 31, 2016. For the purchase period ended August 29, 2014, 24,516 shares of the Company's common stock were purchased for total proceeds of approximately $0.5 million. For the nine months ended September 30, 2014, 46,290 shares of the Company's common stock were purchased for total proceeds of approximately $1.0 million.

Stock-based Compensation Expense

For the three months ended September 30, 2014 and 2013, total stock-based compensation expense was $4.6 million and $3.5 million, respectively. For the nine months ended September 30, 2014 and 2013, total stock-based compensation expense was $14.6 million and $7.5 million, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Research and development	$ 1,668	$ 1,155	$ 5,886	$ 2,409
General and administrative	2,981	2,332	8,692	5,067
Total	$ 4,649	$ 3,487	$ 14,578	$ 7,476

The following table summarizes stock-based compensation expense by grant type included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Stock options	$ 4,217	$ 2,980	$ 13,170	$ 6,413
Restricted stock awards	33	58	169	97
Restricted stock units	—	22	1	250
Stock appreciation rights	147	153	440	442
Employee stock purchase plan	252	274	798	274
Total	$ 4,649	$ 3,487	$ 14,578	$ 7,476

12. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company recorded a net loss for each of the periods presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and are, therefore, excluded from the diluted net loss per share calculation.

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands, except per share amounts)			
Net loss	$ (29,226)	$ (42,030)	$ (91,373)	$ (103,165)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of common stock outstanding for computing basic net loss per share	41,066	33,943	39,595	32,588
Dilutive effect of outstanding warrants and stock awards after application of the treasury stock method*	—	—	—	—
Weighted-average number of common stock outstanding for computing diluted net loss per share	41,066	33,943	39,595	32,588
Net loss per share — basic and diluted	$ (0.71)	$ (1.24)	$ (2.31)	$ (3.17)

* For the three and nine months ended September 30, 2014 and 2013, stock options, restricted stock awards, restricted stock units and stock appreciation rights to purchase approximately 5,305,219 and 5,293,145 shares of common stock were excluded from the net loss per share calculation, respectively, as their effect would have been anti-dilutive.

13. INCOME TAXES

As of December 31, 2013, the Company had gross deferred tax assets of $133.6 million primarily from U.S. federal and state net operating loss ("NOL") carryforwards, U.S. federal and state research and development credit carryforwards, stock-based compensation expense and intangibles. Due to uncertainties surrounding the Company's ability to generate future taxable income to realize these assets, a full valuation allowance has been established to offset its net deferred tax asset. Additionally, the Internal Revenue Code rules could limit the future use of the Company's NOL and research and development credit carryforwards to offset future taxable income based on ownership changes and the value of the Company's common stock.

14. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement ("Cambridge lease") for its headquarters located in Cambridge, Massachusetts. As of September 30, 2014, the Company had entered into four amendments to the Cambridge lease, increasing its total rental space for its headquarters to 69,929 square feet. The Cambridge lease and its amendments will expire in January 2021. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit ("CD") to meet the requirement and it was recorded as a long-term restricted investment in the unaudited condensed consolidated balance sheets as of September 30, 2014.

In June 2014, the Company entered into an agreement to sublease from an unrelated third party 10,939 square feet of office space. The sublease will expire in February 2021.

In January 2014, the Company entered into an agreement to sublease 15,077 square feet of office space to an unrelated third party. The sublease will expire in July 2015.

The Company also leases laboratory and office space in Corvallis, Oregon which will expire in December 2020.

For the three months ended September 30, 2014 and 2013, rent expense and occupancy costs under all leases totaled $1.3 million and $1.0 million, respectively. For the nine months ended September 30, 2014 and 2013, rent expense and occupancy costs under all leases totaled $3.3 million and $2.2 million, respectively.

The aggregate non-cancelable future minimum payments under leases are as follows:

	As of September 30, 2014 (in thousands)
2014 (3 months)	$ 946
2015	4,145
2016	4,320
2017	4,429
2018	4,540
Thereafter	9,932
Total minimum lease payments	$ 28,312

Royalty Obligations

The Company has license agreements for which it is obligated to pay minimum royalties if the Company does not terminate the relevant agreement. The notice period to terminate these agreements is six months or less. For each of the three months ended September 30, 2014 and 2013, royalty payments under these agreements were less than $0.1 million. For each of the nine months ended September 30, 2014 and 2013, royalty payments under these agreements were approximately $0.1 million.

The Company is also obligated to pay royalties upon the net sales of its DMD products. The royalty rates are in the low to mid- single-digit percentages for both inside and outside the United States. For example, under the agreement with Charley's Fund, Inc. signed in October 2007, the Company is obligated to pay a mid-single-digit percentage royalty on the net sales of any product developed pursuant to the agreement with Charley's Fund up to a maximum of $3.4 million. In May 2003, the Company entered into a collaboration and license agreement with Ercole Biotechnology, Inc. and Isis Pharmaceuticals, Inc. ("Isis-Ercole"). The range of percentage of royalty payments under this agreement, should such payments ever be made, is from a fraction of a percent to mid-single-digit percentages.

Milestone Obligations

The Company has license agreements for which it is obligated to pay development and commercial milestones as a product candidate proceeds from the submission of an IND application through approval for commercial sale. There were no significant milestone payments under these agreements for the three or nine months ended September 30, 2014 and 2013, respectively.

Under the collaboration and license agreement with Isis-Ercole, the Company may be obligated to make up to $23.4 million in milestone payments. As of September 30, 2014, the Company had not made any payments under this agreement and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, Isis may be obligated to make milestone payments to the Company of up to $21.1 million in the aggregate for each product developed under a licensed patent under this agreement. As of September 30, 2014, Isis has not made and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied.

In April 2013, the Company and the University of Western Australia ("UWA") entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated. Under the terms of this agreement, UWA granted the Company an exclusive license to certain UWA intellectual property rights in exchange for up to $7.1 million in up-front, development and commercial milestone payments. For the three and nine months ended September 30, 2014 and the three months ended September 30, 2013, the Company did not recognize any expense under this agreement. For the nine months ended September 30, 2013, the Company recognized $1.0 million relating to certain up-front payments required under the agreement as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss.

In March 2014, the Company entered into a patent assignment agreement with a group of scientists (collectively, "Assignors"). Under the terms of the agreement, the Assignors transferred to the Company all rights, title and interest in certain patent rights as well as technical information related to the patents. The Company may be obligated to make up to $2.7 million in development and commercial milestone payments. For the three and nine months ended September 30, 2014, the Company recorded $0 and $0.3 million, respectively, relating to an up-front payment as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (*Corban v. Sarepta, et. al.*, No. 14-cv-10201) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, seeks to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleges that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for DMD, and seeks damages in an unspecified amount. Pursuant to the court's June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and the motion to dismiss is now fully briefed and pending. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

In addition, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al.*, No. 10157). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's CEO was also excessive and such fees were the basis for the CEO not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, amongst others, are disgorgement and rescindment of excessive or unfair payments and equity grants to the CEO and directors, unspecified damages plus interest, a class action declaration for the suit, declaring approval of the Company's Amended and Restated 2011 Equity Plan at the 2013 meeting ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

Purchase Commitments

The Company has entered into long-term contractual arrangements from time to time for the provision of goods and services. The following table presents non-cancelable contractual obligations arising from these arrangements:

	As of September 30, 2014 (in thousands)
2014 (3 months)	$ 11,367
2015	57,729
2016	40,188
2017	23,940
2018	14,260
Thereafter	5,705
Total purchase commitments	$ 153,189

In February 2013, the Company issued two letters of credit totaling $7.3 million to a contract manufacturer in connection with certain manufacturing agreements. The obligations secured by the letters of credit are fulfilled upon payment for certain minimum volume commitments and construction milestones. To meet the requirement of the letters of credit, the Company purchased $7.3 million in CDs. One CD in the amount of $3.3 million was released and matured during the second quarter of 2014 and the other in the amount of $4.0 million with an April 2015 maturity date was recorded as a restricted investment in the unaudited condensed consolidated balance sheets as of September 30, 2014. If the minimum volume commitments have not occurred when the CD matures, the letter of credit will be extended.

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2013 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "seek" and other similar expressions. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our expectations regarding the timing of research, development, preclinical and clinical trial results and analyses relating to safety and clinical benefits of our product candidates including eteplirsen, and our phosphorodiamidate-linked morpholino oligomer ("PMO") chemistries, other RNA-based technology-based chemistries and other RNA-based technology;*
- *our expectations regarding our ability to become a leading developer and marketer of RNA-based therapeutics and commercial viability of our product candidates;*
- *the potential efficacy, potency and utility of our product candidates in the treatment of Duchenne muscular dystrophy ("DMD") and rare and infectious diseases, and their potential to treat a broad number of human diseases;*
- *our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including our ability to effectively manage the clinical trial process for our product candidates, such as our confirmatory studies for eteplirsen;*
- *our ability to timely comply with requirements in connection with development and potential commercialization of our product candidates from applicable regulatory authorities such as the U.S. Food and Drug Administration ("FDA"), including our ability to timely comply with FDA requests and requirements for additional data and analyses in support of our planned submission of a new drug application ("NDA") for eteplirsen and a potential NDA filing, review and approval by the FDA;*
- *Our expectations regarding our ability to timely engage the number of manufacturers with sufficient capability to scale up manufacturing of materials, including subunits, drug substance ("API's") and drug product, within the time frames needed to provide our product candidates to patients in larger scale clinical trials or in potential commercial quantities for eteplirsen and other product candidates and meet regulatory and Company quality control requirements;*
- *the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on us, the development of our product candidates and our financial and contractual obligations;*
- *our expectations regarding the markets for our product candidates;*
- *acceptance of our product candidates, if introduced, in the marketplace;*
- *the possible impact of competitive products, product development, manufacturing, commercialization and technological difficulties;*
- *our expectations regarding partnering opportunities and other strategic transactions;*
- *the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs;*
- *our plans and ability to file additional patent applications to enhance and protect our new and existing technologies and programs;*
- *our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization of our product candidates;*
- *our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Office (the "USPTO") may take with respect to our patent claims or those of third parties, including with respect to interferences that have been declared between our patents and patent applications held by Prosensa Holding N.V. (Prosensa) relating to eteplirsen and SRP-4053 and our expectations regarding the impact of these interferences on our business plans, including our current commercialization plans for eteplirsen;*
- *our ability to operate our business without infringing the intellectual property rights of others;*

- *our estimates regarding how long our currently available cash, cash equivalents and investments will be sufficient to finance our operations and statements about our future capital needs;*
- *the impact of litigation on us, including actions brought by stockholders;*
- *our ability to retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of the loss of key employees;*
- *our expectations relating to the potential for further development of and potential funding from government and other sources for the development of our product candidates, including those targeting Ebola and Marburg viruses; and*
- *other factors set forth below under the heading "Risk Factors".*

These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in this Quarterly Report in Part II, Item 1A — "Risk Factors," and elsewhere in this Quarterly Report. These statements, like all statements in this Quarterly Report, speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. In this report, "we," "our," "us," "Sarepta," and "Company" refers to Sarepta Therapeutics, Inc. and its subsidiaries.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying DMD drug candidates, including our lead product candidate, eteplirsen. We are also developing therapeutics for the treatment of infectious diseases.

Our highly-differentiated RNA-based technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD in the United States. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe but unmet medical need. We are in the process of conducting or starting several studies. These include an ongoing open label extension study following completion of our initial Phase IIb clinical trials, several clinical trials in Exon 51 amenable genotypes, including a confirmatory study in ambulatory patients, studies on participants with early stage and advanced stage DMD and a placebo-controlled confirmatory study with one or more of our follow-on DMD exon-skipping drug candidates. Additionally, we are working on a Phase I/IIa clinical trial for an Exon 53 skipping product candidate in the European Union ("E.U.") and have an open investigational new drug ("IND") application for our Exon 45 skipping product candidate for which we plan to begin a clinical trial early next year. On October 27, 2014, we announced our plans to file an NDA for eteplirsen for the treatment of DMD by mid-year 2015, subject to and pending any additional requests from further discussions with the FDA. We are also leveraging the capabilities of our RNA-based technology platforms to develop therapeutics for the treatment of infectious diseases. We were developing product candidates for Ebola and Marburg under a now expired the Department of Defense ("DoD") contract and further development of these product candidates would be conditioned, in part, on obtaining additional funding, collaborations or emergency use.

The basis for our novel RNA-based therapeutics is our PMO chemistries. Unlike other RNA-based therapeutics, which are often used to down-regulate gene expression, our technologies can be used to selectively up-regulate or down-regulate the production of a target protein, or direct the expression of novel proteins involved in human diseases and disorders. Further, we believe the charge-neutral nature of our PMO-based molecules may have the potential to reduce off-target effects, such as immune stimulatory effects often seen in alternative RNA-based technologies. We believe that our highly-differentiated, novel, proprietary and innovative RNA-based technology platforms, based on charge-neutral morpholino oligomers, may represent a significant improvement over traditional RNA-based technologies.

We have not generated any revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Even if we do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of September 30, 2014, we had approximately $240.7 million of cash, cash equivalents and investments, consisting of $52.3 million of cash and cash equivalents, $183.7 million of short-term investments and $4.6 million of restricted investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months.

Without funding from the U.S. government, we would likely curtail certain infectious disease research and development efforts. We may pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations or license our technology to other companies.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the risks associated with government sponsored programs and the complex regulatory environment in which we operate. There can be no assurance that we will ever achieve significant revenue or profitable operations.

Government Contracts

We recognize revenue from government research contracts and other grants during the period in which the related expenditures are incurred and present the revenue and related expenses gross in the unaudited condensed consolidated financial statements. In the periods presented, substantially all of the revenue generated was derived from government contracts. The following table summarizes the revenue from each of our contracts with the U.S. and E.U. governments for the each of the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
July 2010 Agreement *(Ebola and Marburg IV)*	$ 1,043	$ 2,444	$ 6,816	$ 7,134
August 2012 Agreement (*Intramuscular*)	—	514	—	2,759
European Union SKIP-NMD Agreement *(DMD)*	16	536	1,405	599
July 2013 Children's National Medical Center Agreement *(DMD)*	—	674	659	674
Carolinas Medical Center Agreement (DMD)	—	—	850	—
Other Agreements	—	—	—	427
Total	$ 1,059	$ 4,168	$ 9,730	$ 11,593

July 2010 Agreement (Ebola and Marburg Intravenous administration)

In July 2010, we were awarded the DoD contract managed by its Joint Project Manager Medical Countermeasure Systems ("JPM-MCS") program for the advanced development of our hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003, against Ebola and Marburg viruses, respectively. In February 2012, we announced that we received permission from the FDA to proceed with a single oligomer from AVI-7288, one of the two components that make up AVI-6003, as the lead product candidate against Marburg virus infection. In August 2012, we received a stop-work order related to the Ebola virus portion of the contract and, in October 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The Marburg portion of the contract was structured into four segments and had an aggregate remaining period of performance spanning approximately four years if the DoD exercised its options for all segments. Activities under the first segment began in July 2010 and included preclinical studies and Phase I studies in healthy volunteers. In February 2014, we announced positive safety results from the Phase I multiple ascending dose study of AVI-7288. The contract expired in July 2014. The majority of the revenue under this contract has been recognized as of September 30, 2014 and only revenue for contract finalization, if any, is expected in the future.

For the three months ended September 30, 2014 and 2013, we recognized $1.0 million and $2.4 million, respectively, as revenue under this agreement. For the nine months ended September 30, 2014 and 2013, we recognized $6.8 million and $7.1 million, respectively, as revenue under this agreement.

August 2012 Agreement (Intramuscular)

In August 2012, we were awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, our candidate for treatment of the Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. Accordingly, no revenue was recognized since the conclusion of the contract. For the three and nine months ended September 30, 2013, we recognized $0.5 million and $2.8 million, respectively, as revenue under this agreement.

European Union SKIP-NMD Agreement (DMD)

In November 2012, we entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic. For the three months ended September 30, 2014 and 2013, we recognized less than $0.1 million and slightly more than $0.5 million, respectively, as revenue under this agreement. For the nine months ended September 30, 2014 and 2013, we recognized $1.4 million and $0.6 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of September 30, 2014 and only revenue for contract finalization, if any, is expected in the future.

July 2013 Children's National Medical Center ("CNMC") Agreement (DMD)

In July 2013, we entered into an agreement totaling $1.3 million to provide drug products to CNMC to conduct research related to one of our DMD programs. For the three months ended September 30, 2014 and 2013, $0 and $0.7 million was recognized as revenue under this agreement, respectively. For each of the nine months ended September 30, 2014 and 2013, $0.7 million was recognized as revenue under this agreement. Revenue under this agreement was fully recognized as of March 31, 2014.

Carolinas Medical Center ("CMC") Agreement (DMD)

We entered into a collaboration agreement with CMC to co-develop one of our DMD programs. Under the agreement, CMC was obligated to reimburse certain preclinical costs incurred by us. All preclinical work was completed and we recognized revenue of $0 and $0.9 million for the three and nine months ended September 30, 2014, respectively.

Key Financial Metrics

Revenue

Government Contract Revenue. Substantially all of our revenue has historically been generated from government research contracts and other grants. We recognize revenue from government research contracts and other grants during the period in which the related expenses are incurred and present such revenue and related expenses gross in the unaudited condensed consolidated financial statements.

We defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee that is separate and independent of our performance under the other elements of the arrangement. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of September 30, 2014, we had deferred revenue of $3.4 million, which primarily represents up-front fees which we will recognize as revenue as we satisfy the outstanding performance obligations.

Expenses

Research and Development. Research and development expenses consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities.

Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants and other external services, such as data management and statistical analysis support and materials and supplies used in support of clinical programs. Indirect costs of our clinical programs include salaries, stock-based compensation and an allocation of our facility costs.

Future research and development expenses may also increase as our internal projects, such as eteplirsen for DMD, enter later stage clinical development. Preparations for enrolling patients in a confirmatory trial for eteplirsen are under way and other product candidates are currently in various stages of development. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be ineffective during clinical trials, may take longer to complete clinical trials than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

As a result of these uncertainties and the other risks inherent in the drug development process, we cannot determine the duration or completion costs of current or future clinical stages of any of our product candidates. Similarly, we cannot determine when, if, or to what extent we may generate revenue from the commercialization of any product candidate. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

General and Administrative. General and administrative expenses consist principally of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resource and other general and administrative functions. Other general and administrative expenses include an allocation of our facility costs and professional fees for legal, consulting and accounting services.

Interest Income and Other, Net. Interest income and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income. Our cash equivalents and investments consist of commercial paper, government and government agency bonds, corporate bonds, money market funds and certificates of deposit. Interest expense includes interest paid on our mortgage loan related to the Corvallis property, the substantial portion of which we leased to an unrelated third party in November 2011. Rental income is from subleasing excess space in some of our facilities.

Gain (Loss) on Change in Warrant Valuation. Warrants issued in connection with our January and August 2009 financings were classified as liabilities as opposed to equity due to their settlement terms. These warrants were non-cash liabilities and we were not required to expend any cash to settle these liabilities. The fair value of these warrants was recorded on our unaudited condensed consolidated balance sheets at the date of issuance and the warrants were marked to market at each financial reporting period, with changes in the fair value recorded as "Gain (loss) on change in warrant valuation" in our unaudited condensed consolidated statements of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates related to the inputs used in the model and can result in significant swings in the fair value primarily due to changes in our stock price. As of September 30, 2014, there were no outstanding warrants as all warrants issued in January and August 2009 have been exercised or expired. For more information, please read *Note 7, Warrants* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended September 30, 2014.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States ("U.S. GAAP") requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. For any given individual estimate or assumption we make, there may also be other estimates or assumptions that are reasonable. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time when we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

The policies that we believe are the most critical to aid the understanding of our financial results include:

- revenue recognition;
- research and development expense;
- stock-based compensation; and
- accounting for and valuation of liability classified warrants.

There have been no material changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2013 filed with the Securities and Exchange Commission ("SEC") on March 3, 2014.

Results of Operations for the Three Months and Nine Months Ended September 30, 2014 and 2013

The following table sets forth selected consolidated statements of operations data for each of the periods indicated:

	For the Three Months Ended September 30,		%	For the Nine Months Ended September 30,		%
	2014	2013	Change	2014	2013	Change
	(in thousands, except per share amounts)					
Revenue	$ 1,059	$ 4,168	(75)%	$ 9,730	$ 11,593	(16)%
Operating expenses:						
Research and development	21,852	21,087	4%	63,399	47,833	33%
General and administrative	12,882	8,014	61%	35,398	21,195	67%
Operating loss	(33,675)	(24,933)	35%	(89,067)	(57,435)	55%
Other income (loss):						
Interest income and other, net	193	63	206%	473	281	68%
Gain (Loss) on change in warrant liability	4,256	(17,160)	(125)%	(2,779)	(46,011)	(94)%
Net loss	$ (29,226)	$ (42,030)	(30)%	$ (91,373)	$ (103,165)	(11)%
Net loss per share — basic and diluted	$ (0.71)	$ (1.24)	(43)%	$ (2.31)	$ (3.17)	(27)%

Revenue

Revenue for the three months ended September 30, 2014 decreased by $3.1 million, or 75%, compared with the three months ended September 30, 2013. The decrease was driven by decreases of $1.4 million from the Marburg portion of the July 2010 Agreement which expired in July 2014, $0.7 million from the CNMC Agreement which was completed in the first quarter of 2014, $0.5 million from the August 2012 Agreement which was completed in the third quarter of 2013 and $0.5 million from the decrease in clinical activities of the E.U. SKIP-NMD Agreement.

Revenue for the nine months ended September 30, 2014 decreased by $1.9 million, or 16%, compared with the nine months ended September 30, 2013. The decrease was primarily driven by decreases of $2.8 million from the August 2012 Agreement which was completed in the third quarter of 2013, $0.4 million from a former U.S. government contract related to H1N1 influenza and $0.3 million from the Marburg portion of the July 2010 Agreement which expired in July 2014 partially offset by an increase of $0.8 million from the clinical activities of the E.U. SKIP-NMD Agreement and a $0.9 million reimbursement from CMC on preclinical costs incurred by us for one of our DMD programs.

Research and Development Expenses

Our research and development expenses represent a substantial percentage of our total operating expenses, which primarily consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities. We do not maintain or evaluate and, therefore, do not allocate, internal research and development costs on a project-by-project basis. As a result, a significant portion of our research and development expenses are not tracked by project, as the costs may benefit multiple projects.

The following table summarizes our research and development expenses for each of the periods indicated:

	For the Three Months Ended September 30,		%	For the Nine Months Ended September 30,		%
	2014	2013	Change	2014	2013	Change
	(in thousands)					
DMD	$ 10,604	$ 14,445	(27)%	$ 28,463	$ 26,871	6%
Infectious diseases	37	1,262	(97)%	2,781	4,926	(44)%
Internal research and development costs	11,211	5,380	108%	32,155	16,036	101%
Total research and development expenses	$ 21,852	$ 21,087	4%	$ 63,399	$ 47,833	33%

Research and development expenses for the three months ended September 30, 2014 increased by $0.8 million, or 4%, compared with the three months ended September 30, 2013. The increase was primarily driven by an increase in headcount-related and facility expenses partially offset by a decrease in manufacturing raw materials used.

Research and development expenses for the nine months ended September 30, 2014 increased by $15.6 million, or 33%, compared with the nine months ended September 30, 2013. The increase was primarily driven by preparation for the launch of eteplirsen, should marketing approval ever be obtained, and advancement of our early- and late-stage research and development pipeline. The increase primarily consists of $8.8 million in headcount-related expenses, $1.6 million in our external spend in DMD programs, $2.1 million in external regulatory and manufacturing professional fees, $0.9 million in sponsored research studies and $0.3 million of expense recognized in connection with an up-front payment on a patent assignment agreement. Additionally, there was an increase of $2.3 million in rent and depreciation expenses due to construction of our new headquarters in Cambridge, Massachusetts. The increase was partially offset by an up-front payment of $1.0 million made to University of Western Australia during the second quarter of 2013 and a decrease of $2.1 million in our infectious disease programs.

General and Administrative Expenses

General and administrative expenses for the three months ended September 30, 2014 increased by $4.9 million, or 61%, compared with the three months ended September 30, 2013. The increase was primarily driven by increases of $2.4 million in headcount-related expenses and $2.4 million in professional fees.

General and administrative expenses for the nine months ended September 30, 2014 increased by $14.2 million, or 67%, compared with the nine months ended September 30, 2013. The increase was primarily driven by increases of $6.7 million in headcount-related expenses, $6.0 million in professional fees and $1.2 million in depreciation.

Interest Income and Other, Net

Interest income and other, net, for the three months ended September 30, 2014 increased by $0.1 million compared with the three months ended September 30, 2013. The increase was primarily due to rental income from subleasing excess office space.

Interest income and other, net, for the nine months ended September 30, 2014 increased by $0.2 million compared with the nine months ended September 30, 2013. The increase was primarily due to higher interest income from the increase in our short-term investments balance and rental income from subleasing excess office space.

Gain (Loss) on Change in Warrant Valuation

The gain (loss) on change in warrant valuation changed by $21.4 million from a loss of $17.2 million for the three months ended September 30, 2013 to a gain of $4.3 million for the three months ended September 30, 2014 primarily due to the fluctuation in our stock price and the decrease in the number of our outstanding warrants.

The loss on change in warrant valuation for the nine months ended September 30, 2014 decreased by $43.2 million compared with the nine months ended September 30, 2013 primarily due to the fluctuation in our stock price and the decrease in the number of our outstanding warrants.

As of September 30, 2014, there were no outstanding warrants as all warrants issued in January and August 2009 have been exercised or expired.

For more information, please read *Note 7, Warrants* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended September 30, 2014.

Net Loss

Net loss for the three months ended September 30, 2014 decreased by $12.8 million compared with the three months ended September 30, 2013. The decrease was primarily due to a gain on change in warrant valuation of $4.2 million in 2014 compared to a loss of $17.2 million in 2013. This was partially offset by a decrease of $3.1 million in revenue and an increase of $4.9 million in general and administrative expenses.

Net loss for the nine months ended September 30, 2014 decreased by $11.8 million compared with the nine months ended September 30, 2013. The decrease was primarily due to a decrease of $43.2 million in loss on change in warrant valuation partially offset by increases of $15.6 million in research and development expenses and $14.2 million in general and administrative expenses and a decrease of $1.9 million in revenue.

Liquidity and Capital Resources

As of September 30, 2014, cash, cash equivalents and short-term investments were $236.0 million, compared with $257.0 million at December 31, 2013. The decrease during the nine months ended September 30, 2014 was primarily due to cash used in operating activities of $103.1 million and purchases of property and equipment of $22.3 million. This was partially offset by the public offering of approximately 2.7 million shares which generated net proceeds of $94.5 million and $9.7 million from warrant and option exercises as well as employee stock purchase plan ("ESPP") purchases. Based on the factors described below, we believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months.

Our principal sources of liquidity are the sale of equity securities and revenue from our government contracts and other grants. Our principal uses of cash are research and development expenses, general and administrative expenses, capital expenditures and other working capital requirements.

Our primary source of revenue has historically been from development of product candidates pursuant to government contracts and other grants. U.S. government funding is subject to the government's appropriations process and the government has the right to terminate such contracts for convenience. Our remaining contract with the DoD expired in July 2014. If we do not enter into any additional contracts with or receive funding from the U.S. government, we will likely curtail certain of our infectious disease research and development efforts. Currently, we do not generate any revenue from the commercial sale of our product candidates.

Our future expenditures and capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;
- the timing and costs associated with our clinical trials and preclinical studies;
- the timing and costs associated with commercialization of eteplirsen should marketing approval ever be granted; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technology. We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Nine Months Ended September 30,	
	2014	2013
	(in thousands)	
Cash provided by (used in):		
Operating activities	$ (103,090)	$ (46,873)
Investing activities	(205,707)	(10,850)
Financing activities	104,131	143,706
Decrease in cash and cash equivalents	$ (204,666)	$ 85,983

Operating Activities:

The increase in the amount of cash used in operating activities of $56.2 million for the nine months ended September 30, 2014 compared with the nine months ended September 30, 2013 was primarily due to an increase of $31.6 million in operating loss driven by an increase in research and development and general and administrative expenses and an unfavorable change of $34.9 million in operating assets and liabilities due to the timing of certain activities partially offset by an increase in non-cash adjustments of $10.0 million.

Investing Activities:

The increase in the amount of cash used in investing activities of $194.9 million for the nine months ended September 30, 2014 compared with the nine months ended September 30, 2013 was primarily due to purchase of available-for-sale securities of $272.2 million and an increase of $20.5 million in purchase of property and equipment partially offset by maturity of available-for-sale securities of $86.6 million and a restricted investment of $3.3 million during 2014 and purchase of restricted investments of $7.8 million during 2013.

Financing Activities:

The decrease in the amount of cash provided by financing activities of $39.6 million for the nine months ended September 30, 2014 compared with the nine months ended September 30, 2013 was primarily due to the sale of approximately 3.4 million shares of common stock under the 2013 At-The-Market ("ATM") offering which generated net proceeds of $123.0 million, the sale of approximately 87,000 shares of common stock under the 2012 ATM offering which generated net proceeds of $2.1 million and decrease of $10.2 million in net proceeds from warrant and stock option exercises partially offset by the 2014 public offering of approximately 2.7 million shares which generated net proceeds of $94.5 million and $1.0 million from the ESPP purchases.

Contractual Obligations and Contingencies

In our continuing operations, we have entered into long-term contractual arrangements for our facilities, the provision of goods and services and acquisition of technology access rights, among others. The following table presents non-cancelable contractual obligations arising from these arrangements as of September 30, 2014:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
	(in thousands)				
Long-term debt(1)	$ 2,122	$ 171	$ 343	$ 343	$ 1,265
Notes payable(1)	5,019	2,515	2,504	—	—
Operating leases	28,312	4,043	8,690	9,136	6,443
Purchase obligations(2)	153,189	64,819	66,980	21,390	—
Total	$188,642	$ 71,548	$ 78,517	$ 30,869	$ 7,708

(1) Interest is included.
(2) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding to us and that specify all significant terms. Purchase obligations relate primarily to our DMD development program.
(3) We are obligated to make up to $32.4 million of future development and commercial milestone payments associated with some of our collaboration and license agreements.

Off-Balance Sheet Arrangements

During the periods presented, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or for another contractually narrow or limited purpose.

Recent Accounting Pronouncements

For additional information, please read *Note 2, Recent Accounting Pronouncements* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended September 30, 2014.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, government and government agency bonds and high-grade corporate bonds with maturities of twenty-four months or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of September 30, 2014, we had $240.7 million of cash, cash equivalents and investments, comprised of $52.3 million of cash and cash equivalents, $183.7 million of short-term investments and $4.6 million of restricted investments. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of September 30, 2014, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1 million to our interest rate sensitive instruments.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this report, Form 10-Q for the quarterly period ended September 30, 2014, under the supervision and with the participation of our management, including our chief executive officer and our chief financial officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (the "Exchange Act"). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (ii) is accumulated and communicated to our management, including our chief executive officer and our chief financial officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2014, our disclosure controls and procedures were effective.

Changes in Internal Control Over Financial Reporting

As of December 31, 2013, a material weakness in our internal controls over financial reporting was identified as follows: The Company did not design and implement controls to adequately review and consider the recognition and measurement of new significant research and development contracts. During the nine months ended September 30, 2014, we designed and implemented controls to adequately review and consider the recognition and measurement of new significant research and development contracts. Such contracts are timely reviewed by our accounting personnel with the requisite accounting knowledge, skills and experience deemed necessary to perform such a review.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (*Corban v. Sarepta, et. al.*, No. 14-cv-10201) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, seeks to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleges that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for DMD, and seeks damages in an unspecified amount. Pursuant to the court's June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and the motion to dismiss is now fully briefed and pending. In addition, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al.*, No. 10157). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's Chief Executive Officer ("CEO") was also excessive and such fees were the basis for the CEO not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, amongst others, are disgorgement and rescindment of excessive or unfair payments and equity grants to the CEO and directors, unspecified damages plus interest, a class action declaration for the suit, declaring approval of the Company's Amended and Restated 2011 Equity Plan at the 2013 meeting ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

Item 1A. Risk Factors.

Factors That Could Affect Future Results

Set forth below and elsewhere in this report and in other documents we file with the Securities and Exchange Commission ("SEC") are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Relating to Our Business

Most of our product candidates are at an early stage of development and may never receive regulatory approval.

With the exception of eteplirsen, which is being studied in a confirmatory clinical trial, our product candidates are in relatively early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, eteplirsen in Duchenne muscular dystrophy ("DMD") and AVI-7100 in influenza are in active clinical development. We are working on a Phase I/IIa clinical trial to study an Exon 53 skipping product candidate in the European Union. We now have an open investigational new drug ("IND") for our Exon 45 skipping product candidate and plan to begin a clinical study early next year. AVI-7537 in Ebola and AVI-7288 in Marburg were being developed through a program with the U.S. Department of Defense ("DoD") and further development is conditioned in part on obtaining additional funding, collaborations or emergency use. Our other product candidates are in preclinical development or inactive. We expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with eteplirsen and other exon-skipping candidates as part of our larger pan-exon strategy in DMD, our infectious disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates and successfully obtain approvals needed to market them.

Our RNA-based antisense technology has not been incorporated into a therapeutic commercial product and is still at a relatively early stage of development.

Our RNA-based platforms, utilizing proprietary Phosphorodiamidate-linked morpholino oligomer ("PMO") based technology, have not been incorporated into a therapeutic commercial product and are still at a relatively early stage of development. This technology is used in all of our product candidates, including eteplirsen. Although we have conducted clinical studies with eteplirsen and preclinical studies with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in preclinical studies. Any failures or setbacks in developing or utilizing our PMO-based technology, including adverse effects in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial position.

We have been granted orphan designations in the U.S. and in the E.U. for certain of our product candidates, however, there can be no guarantee that we will receive orphan status for these product candidates nor that we will be able to prevent third parties from developing and commercializing products that are competitive to these product candidates.

To date we have been granted orphan drug designation under the Orphan Drug Act by the U.S. Food and Drug Administration ("FDA") for two of our product candidates in DMD (including eteplirsen), AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of the Marburg virus. Upon approval from the FDA of a new drug application ("NDA"), products granted orphan drug status are generally provided with seven years of marketing exclusivity in the United States, meaning the FDA will generally not approve applications for other product candidates for the same orphan indication that contain the same active ingredient. Even if we

are the first to obtain approval of an orphan product and are granted exclusivity in the United States, there are limited circumstances under which a later competitor product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our product or due to an inability to assure a sufficient quantity of the orphan drug.

To date we have been granted orphan medicinal product designations in the European Union for two of our product candidates in DMD (including eteplirsen). Product candidates granted orphan status in Europe can be provided with up to 10 years of marketing exclusivity, meaning that another application for marketing authorization of a later similar medicinal product for the same therapeutic indication will generally not be approved in Europe. Although we may have drug candidates that may obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period may be modified for several reasons, including a significant change to the orphan medicinal product designations or status criteria after market authorization of the orphan product (*e.g.*, product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

We are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the United States or the European Union, our business and results of operations could be materially adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the United States and the European Union for the time periods specified above, we would not be able to exclude other companies from manufacturing and/or selling products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. Moreover, we cannot guarantee that another company will not receive approval before we do of an orphan drug application in the United States or the European Union for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our drug candidates for which we plan to file for orphan designation and status. If that were to happen, our orphan drug applications for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the United States or the European Union, as applicable. Further, application of the orphan drug regulations in the United States and Europe is uncertain, and we cannot predict how the respective regulatory bodies will interpret and apply the regulations to our or our competitors' product candidates.

Even if we receive regulatory approvals for any of our product candidates it is possible that they may not become commercially viable products.

Even if a product candidate receives regulatory approval, the product may not gain market acceptance among physicians, patients, healthcare or third-party payers and the medical community which could limit commercialization of the product. Assuming that any of our product candidates receives the required regulatory approvals, commercial success will depend on a number of factors, including but not limited to the following:

- demonstration and/ or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;
- cost-effectiveness of the product;
- the availability of adequate reimbursement by third parties, including government payers such as the Medicare and Medicaid programs, managed care organizations and private health insurers;
- the product's potential advantage over alternative or competitive treatment methods;
- whether the product can be manufactured in commercial quantities and at acceptable costs;
- marketing and distribution support for the product;
- any exclusivities or patent rights applicable to the product; and
- our ability to achieve and sustain profitability, which may not occur if we are unable to develop and commercialize any of our product candidates, development is delayed or sales revenue from any product candidate that receives marketing approval is insufficient.

If there are significant delays in obtaining or we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all, which would materially impair our ability to generate revenue and have a successful business.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the United States, approvals and oversight from federal (e.g. FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the United States or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates, including eteplirsen, on an accelerated approval or any other basis, in any jurisdiction, including in the United States, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our preclinical, clinical, Chemistry, Manufacturing and Controls ("CMC") and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for submissions, filings or approvals. For example, the FDA could disagree with our beliefs, interpretations and conclusions regarding data in an NDA submission for eteplirsen, or other product candidates, and may reject or refuse to file our planned NDA submission until we meet their additional requirements, if ever. Even if we meet such requirements and our NDA is accepted for review or filed, the FDA could still deny approval of eteplirsen, or other product candidates, based on their review of the data or other factors.
- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and novel surrogate endpoints, such as dystrophin measures, is uncertain due to the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. As a result we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned INDs and NDAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (*e.g.*, additional patient muscle biopsies and dystrophin analysis), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, even if initially accepted, regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Responding to requests from regulators and meeting requirements for submissions, filings and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third-party vendors and associates may be complicated by our own limitations and those of the parties we work with. For example, changes to CMC processes for the production of eteplirsen may require coordination with our third-party manufacturers, which may or may not be limited in their abilities to execute such regulatory requests. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including related to clinical trial design and the timing of NDA filings.

Due to the above factors, among others, our product candidates could take a significantly longer time to gain regulatory approval than we expect, or may never gain regulatory approval, which could delay or eliminate any potential commercialization or product revenue for us. Even if we are able to comply with all regulatory requests and requirements, the delays resulting from satisfying such requests and requirements, the cost of compliance, or the effect of regulatory decisions (*e.g.*, limiting labeling and indications requested by us for a product candidate) may no longer make commercialization of a product candidate desirable for us from a business perspective, which could result in a Company decision to not commercialize a product candidate.

Even after approval and commercialization of a product candidate, we would remain subject to ongoing regulatory compliance and oversight to maintain our approval. If we are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties or we may not be permitted to continue marketing our products, which could have a material adverse effect on our financial condition and harm our competitive position in the market place.

Our preclinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive preclinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future preclinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in preclinical and early clinical trials does not ensure that the subsequent trials we plan to conduct will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld. For example, in 2012, we completed Study 201, a U.S.-based Phase IIb 12-person clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. Following completion of this study, we initiated Study 202, an ongoing open label extension study with the same participants from Study 201. These trials were initiated, in part, to further demonstrate efficacy and safety, including the production of dystrophin, and explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. While Studies 201 and 202 met their primary endpoints of dystrophin production at weeks 24 and 48, respectively, and six-minute walk test results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot provide assurances that data from the ongoing open label extension study will continue to be positive or consistent through the study periods. For example, on July 10, 2014, we announced the results for week 144 in Study 202, which showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history; however, the decline on the six-minute walk test from baseline, although in prior study results was below 5%, was measured at approximately 8.5%. If the data from the confirmatory studies for eteplirsen do not produce the safety and efficacy data required by regulatory authorities for an NDA submission, filing or approval, we may need to continue working with the FDA on the design and subsequent execution of any further studies or analysis we plan to conduct or that may be required for the approval of eteplirsen or our other DMD product candidates. For example, in October 2014, we received meeting minutes from a Type B Pre-NDA meeting that took place in September 2014 in which the FDA provided updated guidance regarding the information to be provided as part of, or at the time of, our planned NDA submission for eteplirsen. The guidance stated that the FDA was requiring additional data as part of the NDA submission, including the results from an independent assessment of dystrophin images and the 168 week clinical data from Study 202. Additionally, the guidance requested more specific data, such as a minimum duration of safety in new patients exposed to eteplirsen, patient-level natural history data to be obtained by Sarepta from independent academic institutions and MRI data from a recent study conducted by an independent group. The FDA also indicated that further discussion would be needed to determine what would constitute a complete NDA submission.

We currently rely on third parties in the manufacturing process to produce our product candidates and our dependence on these parties, or our inability to engage sufficient third parties to meet manufacturing needs for large-scale clinical trials or potential commercial needs within sufficient timelines, may impair the advancement of our research and development programs and potential commercialization of our product candidates.

We do not currently have the internal ability to undertake the manufacturing process for our product candidates in the quantities needed to conduct our research and development programs, supply clinical trials or meet commercial demand. We therefore rely on, and expect for the foreseeable future to continue relying on, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), drug substance ("API") and drug product, as well as perform additional steps in the manufacturing process, such as the filling and labeling of vials and storage of our product candidates.

There are a limited number of third parties with facilities and capabilities required for the manufacturing process of our product candidates which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. Any interruption of the development or operation of those facilities due to, amongst other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available product candidates or materials.

If these third parties were to cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to have our product candidates manufactured in sufficient quality and quantities required for planned preclinical testing, clinical trials and potential commercial use would be adversely affected.

We have not engaged or contracted with all the third parties needed for the production of materials and APIs for any of our product candidates, including eteplirsen, in quantities sufficient for their potential commercial demand or for multiple large-scale clinical trials. In light of the limited number of third parties with the expertise to produce our product candidates, and the underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our product candidates. Further, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our product candidates, such parties may not comply with the terms and

time-lines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required time-lines in connection with the regulatory approval process and potential commercialization. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties which could have a material adverse effect on our business prior to and after commercialization of any our product candidates.

The manufacturing process for our product candidates may fail to comply with cGMP standards.

Our contract manufacturers are required to produce our materials, APIs and drug products under current Good Manufacturing Practice ("cGMP") standards. We and our contract manufacturers are subject to periodic unannounced inspections by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations and corresponding foreign standards. We do not have control over a third-party manufacturer's compliance with these regulations and standards. In addition, changes in cGMP standards could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our product candidates in a compliant manner on the schedule we require for clinical trials or for potential commercial use. The failure to achieve and maintain high quality standards, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in patient injury or death or product recalls. Any difficulties or delays in our contractors' manufacturing and supply of product candidates, or any failure of our contractors to maintain compliance with the applicable regulations and standards could increase our costs, make us postpone or cancel clinical trials, prevent or delay regulatory approvals by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, result in the termination of the development of a product candidate, or have our product candidates recalled or withdrawn from use.

We may not be able to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could delay or prevent us from developing or commercializing our product candidates.

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for larger and later stage clinical trials for our product candidates, including eteplirsen, and potential commercialization, we are working to increase the manufacturing capacity and scale up production of some of the components of our drug products. During 2014, we will continue to increase material and API production capacity to provide the drug product needed for additional eteplirsen trials and studies for our other product candidates (including a placebo-controlled study planned for one or more of our follow-on exon product candidates) and any planned subsequent commercialization, on an accelerated or other pathway. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third-party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory standards or is cost-effective, or in a time frame required to meet our timelines for clinical trials, potential commercialization and other business plans, or at all. cGMP and other quality issues may arise during our efforts to increase manufacturing capacity and scale-up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of a product candidate itself or the product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release product and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our analytical methods or demonstrate adequate purity, stability or comparability of the product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our analytical methods or to demonstrate adequate purity, stability or comparability, the development of our product candidates and regulatory approval or commercial launch for any resulting drug products may be delayed, which could significantly harm our business.

During our work with our third-party manufacturers to increase manufacturing capacity and scale-up production, it is possible that they could make improvements in the manufacturing and scale-up processes for our product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, it is possible that we will need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Any failure to secure the intellectual rights required for the manufacturing process needed for large-scale clinical trials or commercialization of our product candidates could cause significant delays in our business plans or could prevent commercialization of our product candidates.

We are currently winding down our expired U.S. government contract and further development of Ebola and Marburg product candidates may be limited by our ability to obtain additional funding for these programs and by the intellectual property and other rights retained by the U.S. government.

We have historically relied on U.S. government contracts and awards to fund and support certain development programs, including our Ebola and Marburg programs. The July 2010 DoD contract providing funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits and other government requirements successfully, the government may withhold some or all of the currently outstanding amounts owed to us.

We are currently exploring and evaluating options to continue advancing the development of our Ebola and Marburg product candidates, which may or may not include funding through U.S. government programs. As a result of government budgetary cuts, appropriations and sequestration, amongst other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The option for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates we have developed under a U.S. government contract after such contract is terminated or expired.

We may not be able to successfully conduct clinical trials due to various process-related factors which could negatively impact our business plans.

The successful start and completion of any of our clinical trials within time frames consistent with our business plans is dependent on various factors, which include, but are not limited to, our ability to:

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients to power a clinical trial:
  - Participant enrollment and retention is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy or personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations ("CROs") involved in the clinical trial:
  - Negotiating contracts and other related documents with clinical trial parties and independent review boards ("IRBs"), such as informed consents, CRO agreements and site agreements, can be subject to extensive negotiations that could cause significant delays in the clinical trial process. In addition, terms may vary significantly among different trial sites and CROs and may subject the Company to various risks;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;
- conduct the clinical trials in a cost effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

If we are not able to manage the clinical trial process successfully, our business plans could be delayed or be rendered unfeasible for us to execute within our planned or required time frames, or at all.

We have incurred operating losses since our inception and we may not achieve or sustain profitability.

We incurred an operating loss of $33.7 million for the nine months ended September 30, 2014. Our accumulated deficit was $634.6 million as of September 30, 2014. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any material revenue from product sales and have incurred expenses related to research and development of our technology and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure and acquired in-process research and development resulting from two acquisitions. We anticipate that our expenses will increase substantially if and as we:

- continue our research, preclinical and clinical development of our product candidates;

- responding to and satisfying requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- acquire or in-license other product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Our ability to achieve and maintain profitability depends on our ability to raise additional capital, partner one or more programs, complete development of our product candidates, obtain regulatory approvals and market our approved products, if any. It is uncertain when, if ever, we will become profitable and if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the Company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

We will need additional funds to conduct our planned research, development and manufacturing efforts. If we fail to attract significant capital or fail to enter into strategic relationships, we may be unable to continue to develop our product candidates.

We will likely require additional capital from time to time in the future in order to continue the development of product candidates in our pipeline and to expand our product portfolio. The actual amount of funds that we may need will be determined by many factors, some of which are beyond our control. These factors include the success of our research and development efforts, the status of our preclinical and clinical testing, costs and timing relating to securing regulatory approvals and obtaining new patent rights, regulatory changes, competitive and technological developments in the market and future commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

We would expect to seek additional financing from the sale and issuance of equity or equity-linked or debt securities, and we cannot predict that financing will be available when and as we need financing or that, if available, the financing terms will be commercially reasonable. If we are unable to obtain additional financing when and if we require it or on commercially reasonable terms, this would have a material adverse effect on our business and results of operations.

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. For example, on April 29, 2014, we sold 2,650,000 shares of our common stock in an underwritten public offering at a price to the public of $38.00 per share. 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.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than preclinical collaborations with academic/research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD and a product candidate for the treatment of influenza, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates. Such relationships may require us 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.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our condensed consolidated financial statements could prove inaccurate.

Our condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include those related to revenue recognition, accrued expenses, assumptions in the valuation of stock-based compensation and accounting for and valuation of liability classified warrants. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our financial statements, which could in turn subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited as a result of future transactions involving our common stock.

In general, under Section 382 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an ‘‘ownership change’’ is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders’ lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such “ownership change.” Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we have estimated would otherwise be required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

We rely on third parties to provide services in connection with our preclinical and clinical development programs. The inadequate performance by or loss of any of these service providers could affect our product candidate development.

Several third parties provide services in connection with our preclinical and clinical development programs, including in vitro and in vivo studies, assay and reagent development, immunohistochemistry, toxicology, pharmacokinetics, clinical assessments, data monitoring and management, statistical analysis and other outsourced activities. If these service providers do not adequately perform the services for which we have contracted or cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our product candidates, our development programs may be delayed.

If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth and our ability to compete would suffer.

We are highly dependent on the efforts and abilities of the principal members of our senior management. Additionally, we have scientific personnel with significant and unique expertise in RNA-based therapeutics and related technologies. The loss of the services of any one of the principal members of our managerial team or staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain and motivate such personnel. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

If we are unable to effectively manage our growth, execute our business strategy and implement compliance controls and systems, the trading price of our common stock could decline. Although we did not have a material error in our financial statements, we have identified a material weakness in our internal control over financial reporting as of December 31, 2013. Any ongoing failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

We anticipate continued growth in our business operations due, in part, to advancing our product candidates. This future growth could create a strain on our organizational, administrative and operational infrastructure. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to build the management and human resources and infrastructure necessary to support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete this in a timely and efficient manner could adversely affect our operations.

For example, although there was no material error in our financial statements, in connection with our assessment of the effectiveness of internal control over financial reporting as of December 31, 2013, our management identified a material weakness in our internal control over financial reporting. A detailed description of this material weakness is provided in "Item 9A, Controls and Procedures" of our annual report on Form 10-K filed earlier this year. During the nine months ended September 30, 2014, we designed and implemented controls to adequately review and consider the recognition and measurement of new significant research and development contracts. Our controls were designed to ensure that such contracts are timely reviewed by our accounting personnel with the requisite accounting knowledge, skills and experience deemed necessary to perform such a review. However, we cannot provide assurances that material weaknesses in our internal control over financial reporting will not be identified in the future. Any failure to maintain or implement new or improved internal controls, or any difficulties that we may encounter in their maintenance or implementation, could result in additional material weaknesses or material misstatements in our financial statements and cause us to fail to meet our reporting obligations or prevent fraud, which could cause the trading price of our common stock to decline.

We may not be able to build the human resources and infrastructure necessary to support the growth of our business or to appropriately implement our compliance controls and procedures. The time and resources required to build up our human resources and implement systems and infrastructure that may be needed to support our growth and compliance with applicable rules and regulations is uncertain, and failure to complete these in a timely and efficient manner could adversely affect our operations.

We may engage in future acquisitions or collaborations with other entities that increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring complementary products, technologies or businesses. Potential acquisitions or collaborations with other entities may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products, retention of key employees, diversion of our management's attention and uncertainties in our ability to maintain key business relationships of the acquired entities. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our product candidates, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties.

We currently hold various issued patents and exclusive rights to issued patents and own and have licenses to various patent applications, in each case in the United States as well as rights under European patents and patent applications. We anticipate filing additional patent applications both in the United States and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. The risks we face on the intellectual property front include the following:

- We may not be able to obtain and maintain patent protection for our product candidates that has the ability to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise might not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the "PTAB") of the United States Patent and Trademark Office (the "USPTO") declared patent interferences between certain patents held by Sarepta and patent applications held by Prosensa related to Exon 51 and Exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies

Sarepta's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering eteplirsen, as interfering with Prosensa's U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta's U.S. Patent No. 8,455,636, covering SRP-4053, as interfering with Prosensa's U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the Exon 51 skipping therapies that are the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta's U.S. Patent No. 8,486,907, which covers certain methods of using eteplirsen, as interfering with Prosensa's U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB requesting the declaration of a fourth interference relating to certain methods concerning the Exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta's U.S. Patent No. 8,455,636 and Prosensa's U.S. Application No. 14/248,279. These interferences do not currently change our plans to submit an NDA for eteplirsen, continue with our clinical development plans for eteplirsen and SRP-4053 or our ability to launch eteplirsen commercially if it is approved by the FDA under an accelerated approval pathway, and patents held or licensed to Sarepta and included in these interference proceedings are presumed valid by statute for the duration of these proceedings and any appeals. However, if final resolution of the interferences and related appeals, if any, are not in our favor, then these patents and any other patents or applications also found to be interfering may be invalidated, and as a result, we may not have any patent-based exclusivity available for our product candidates, which may have a negative impact on our business plan. Failure to resolve the interferences or related appeals in our favor could also result in the grant of patent claims to Prosensa that could provide a basis to assert that commercialization of eteplirsen and SRP-4053 in the United States infringe on such claims. These interferences may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, Prosensa may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the United States and in other jurisdictions;

- As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful.
- Jurisdictions other than the United States might have less restrictive patent laws than the United States, giving foreign competitors the ability to exploit these laws to create, develop and market competing products.

In addition, the USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Additionally, the full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc*., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics, Inc*., the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated natural products is uncertain and, as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Our business prospects will be impaired if third parties successfully assert that our product candidates or technologies infringe proprietary rights of such third parties.

Our competitors may make significant investments in competing technologies, and might have or obtain patents that limit, interfere with or eliminate our ability to make, use and sell our product candidates in important commercial markets.

If our product candidates or technologies infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate;
- redesign product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition and operations. Prosensa, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. Prosensa has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office ("EPO"), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of eteplirsen in Europe would infringe on such patent. Both we and Prosensa have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other's briefs. Prosensa recently filed arguments with the EPO in response to Sarepta's previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial effect on our business and leaves open the possibility that Prosensa or other parties that have rights to such patent could assert that our drug eteplirsen infringes on such patent. The timing and outcome of appeal cannot be predicted or determined as of the date of this report. We are also aware of existing patent claims Prosensa is pursuing in the United States, including those involved in the interferences declared by the USPTO in July 2014 and September 2014 and discussed in these risk factors, and others that it has or is pursuing in other countries, that where granted may provide the basis for Prosensa or other parties to assert that commercialization of eteplirsen and certain other of our product candidates would infringe on such claims.

The DMD patent landscape is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

We face intense competition and rapid technological change, which may result in others discovering, developing or commercializing competitive products.

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas related to antisense technology and other RNA technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Isis Pharmaceuticals, Inc., SantarisPharma A/S and Nippon Shinyaku Co. Ltd. share a focus on RNA-based drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include Prosensa and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs.

Although Prosensa/GlaxoSmithKline plc announced in 2013 that the primary endpoint for their lead DMD drug candidate was not met, we may still face competitive risks arising from the Prosensa exon skipping platform and product candidate pipeline, which may include limitations on our ability to gain market share in the DMD space or other diseases targeted by our exon skipping platform and product candidate pipeline.

Other potential competitors include large, fully integrated pharmaceutical companies and more established biotechnology companies that have significantly greater resources and expertise in research and development, manufacturing, testing, obtaining regulatory approvals and marketing. Also, academic institutions, government agencies and other public and private research organizations conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and marketing. It is possible that these competitors will succeed in developing technologies that are more effective than our product candidates or that would render our technology obsolete or noncompetitive. Our competitors may, among other things:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

We may be subject to product liability claims and our insurance may not be adequate to cover damages.

We currently have no products that have been approved for commercial sale; however, the current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of any products in the future, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for any of our product candidates. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Our operations involve the use of hazardous materials, and we must comply with environmental laws, which can be expensive, and may affect our business and operating results.

Our research and development activities involve the use of hazardous materials, including organic and inorganic solvents and reagents. Accordingly, we are subject to federal, state and local laws and regulations governing the use, storage, handling, manufacturing, exposure to and disposal of these hazardous materials. In addition, we are subject to environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Although we believe that our activities conform in all material respects with such environmental laws, there can be no assurance that violations of these laws will not occur in the future as a result of human error, accident, equipment failure or other causes. Liability under environmental, health and safety laws can be joint and several and without regard to fault or negligence. The failure to comply with past, present or future laws could result in the imposition of substantial fines and penalties, remediation costs, property damage and personal injury claims, loss of permits or a cessation of operations, and any of these events could harm our business and financial condition. We expect that our operations will be affected by other new environmental, health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur a liability and our research and development programs and the development of our product candidates could be delayed.

We may incur substantial costs in connection with litigation and other disputes.

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, and employee matters. It is possible that we may not prevail in claims made against us in such disputes even after expending significant amounts of money and company resources in defending our positions in such lawsuits and disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

Risks Related to Our Common Stock

Our stock price is volatile and may fluctuate due to factors beyond our control.

The market prices for and trading volumes of securities of biotechnology companies, including our securities, have been historically volatile. Historically, our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the past year, in a single day, our stock has increased as much as 64% in a single day or decreased as much as 39% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the timing of our submissions to regulatory authorities and regulatory decisions and developments including any potential decision by the FDA to review eteplirsen on an expedited or normal pathway, if at all;
- positive or negative results from or regulatory interpretations of testing and clinical trials by ourselves, strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by our product candidates;
- delays in beginning and completing preclinical and clinical studies for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- technological innovations or commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- financing, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation such as the stockholder lawsuits against us; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of companies' stock, including ours, regardless of actual operating performance. In addition, in the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management's attention and resources.

Provisions of our certificate of incorporation, bylaws and Delaware law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the then-current management and board of directors.

Certain provisions of our certificate of incorporation and bylaws may make it more difficult for a third party to acquire control of us or effect a change in our board of directors and management. These provisions include:

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of majority of the voting power of all the then-outstanding shares of voting stock;

- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These and other provisions in our certificate of incorporation and our bylaws and in the Delaware General Corporation Law could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors.

We expect our operating results to fluctuate in future periods, which may adversely affect our stock price.

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing of activities performed in support of our U.S. government research contracts and the timing and magnitude of expenditures incurred in support of our DMD and other proprietary drug development programs. In one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards and warrants, and we expect to issue additional stock awards and shares of common stock in the future. Exercise of these awards, and sales of shares, will dilute the interests of existing security holders and may depress the price of our common stock.

As of September 30, 2014, there were 41.3 million shares of common stock outstanding, outstanding awards to purchase 5.3 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2014, there were 1.9 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and 0.9 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock, perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report on Form 10-Q.

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.

SAREPTA THERAPEUTICS, INC.

Date: November 6, 2014

By: /s/ CHRISTOPHER GARABEDIAN

Christopher Garabedian
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 6, 2014

By: /s/ SANDESH MAHATME

Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
31.1	Certification of the Company's President and Chief Executive Officer, Christopher Garabedian, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's President and Chief Executive Officer, Christopher Garabedian, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

* The Certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filings of Sarepta Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

Exhibit 31.1

CERTIFICATION

I, Christopher Garabedian, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc. (the "Registrant");

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

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

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

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

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

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

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

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

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

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

November 6, 2014

/s/ Christopher Garabedian

Christopher Garabedian
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Sandesh Mahatme, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc. (the "Registrant");

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

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

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

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

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

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

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

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

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

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

November 6, 2014

/s/ Sandesh Mahatme

Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Christopher Garabedian, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2014, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 6, 2014

/s/ Christopher Garabedian

Christopher Garabedian,
President and Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

Exhibit 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2014, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 6, 2014

/s/ Sandesh Mahatme

Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.