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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2013

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 7, Cambridge, Massachusetts	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (857) 242-3700

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	**37,582,309**
(Class)	**(Outstanding as of October 31, 2013)**

SAREPTA THERAPEUTICS, INC.
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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

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

	September 30, 2013	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$ 273,644	$ 187,661
Accounts receivable	6,721	4,713
Restricted investments	7,250	—
Other current assets	2,807	1,534
Total current assets	290,422	193,908
Restricted investments	557	—
Property and equipment, net of accumulated depreciation and amortization of $17,345 and $16,708	8,244	3,397
Patent Costs, net of accumulated amortization of $1,538 and $2,626	5,231	4,913
Other assets	25	2,775
Total assets	$ 304,479	$ 204,993
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,129	$ 7,532
Accrued employee compensation	2,909	2,741
Current portion of long-term debt	91	89
Warrant liability	35,994	65,193
Deferred revenue	3,963	3,304
Other current liabilities	15	27
Total current liabilities	51,101	78,886
Long-term debt	1,599	1,668
Other long-term liabilities	4,795	760
Total liabilities	57,495	81,314
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; Issued and outstanding: 37,533,695 at September 30, 2013 and 31,703,817 at December 31, 2012	4	3
Additional paid-in capital	781,396	554,927
Deficit accumulated during the development stage	(534,416)	(431,251)
Total stockholders' equity	246,984	123,679
Total liabilities and stockholders' equity	$ 304,479	$ 204,993

See accompanying notes to condensed consolidated financial statements.

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

	Three months ended September 30,		Nine months ended September 30,		July 22, 1980 (Inception) through
	2013	2012	2013	2012	September 30, 2013
Revenues from license fees, grants and research contracts	$ 4,168	$ 7,574	$ 11,593	$ 29,993	$ 185,141
Operating expenses:					
Research and development	21,087	10,914	47,833	39,568	433,501
General and administrative	8,014	3,565	21,195	9,761	140,282
Acquired in-process research and development	—	—	—	—	29,461
Operating loss	(24,933)	(6,905)	(57,435)	(19,336)	(418,103)
Other (loss) income:					
Interest income and other, net	63	67	281	270	9,804
Loss on change in warrant valuation	(17,160)	(42,716)	(46,011)	(40,154)	(112,979)
Realized gain on sale of short-term securities — available-for-sale	—	—	—	—	3,863
Write-down of short-term securities — available-for-sale	—	—	—	—	(17,001)
Total other loss	(17,097)	(42,649)	(45,730)	(39,884)	(116,313)
Net loss	$ (42,030)	$(49,554)	$(103,165)	$(59,220)	$ (534,416)
Other comprehensive (loss) income:					
Write-down of short-term securities — available-for-sale	—	—	—	—	17,001
Realized gain on sale of short-term securities — available-for-sale	—	—	—	—	(3,863)
Unrealized loss on short-term securities — available-for-sale	—	—	—	—	(13,138)
Total other comprehensive (loss) income	—	—	—	—	—
Comprehensive loss	$ (42,030)	$(49,554)	$(103,165)	$(59,220)	$ (534,416)
Net loss per share — basic	$ (1.24)	$ (2.17)	$ (3.17)	$ (2.61)	
Net loss per share — diluted	$ (1.24)	$ (2.17)	$ (3.17)	$ (2.61)	
Weighted average number of common shares outstanding for computing basic net loss per share	33,943	22,824	32,588	22,691	
Weighted average number of common shares outstanding for computing diluted net loss per share	33,943	22,824	32,588	22,691	

See accompanying notes to condensed consolidated financial statements.

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

	Nine months ended September 30,		For the Period July 22, 1980 (Inception) through
	2013	2012	September 30, 2013
Cash flows from operating activities:			
Net loss	$ (103,165)	$ (59,220)	$ (534,416)
Adjustments to reconcile net loss to net cash flows used in operating activities:			
Depreciation and amortization	998	1,090	22,968
Loss on disposal of property and equipment	460	182	3,096
Realized gain on sale of short-term securities — available-for-sale	—	—	(3,863)
Write-down of short-term securities — available-for-sale	—	—	17,001
Impairment charge on real estate owned	—	—	1,445
Stock-based compensation	7,476	1,840	39,549
Acquired in-process research and development	—	—	29,461
Increase on warrant liability	46,011	40,154	112,979
Changes in operating assets and liabilities:			
Net increase in accounts receivable, other current assets and other assets	(520)	(1,162)	(9,281)
Net increase (decrease) in accounts payable, accrued employee compensation, and other liabilities	1,867	(5,420)	14,280
Net cash used in operating activities	(46,873)	(22,536)	(306,781)
Cash flows from investing activities:			
Purchase of restricted investments	(7,807)	—	(7,807)
Purchase of property and equipment	(1,762)	(108)	(21,749)
Patent costs	(1,281)	(614)	(11,810)
Purchase of marketable securities	—	—	(112,993)
Sale of marketable securities	—	—	117,724
Acquisition costs	—	—	(2,389)
Net cash used in investing activities	(10,850)	(722)	(39,024)
Cash flows from financing activities:			
Proceeds from sale of common stock, warrants, and partnership units, net of offering costs, and exercise of options and warrants	143,951	21,405	620,510
Repayments of long-term debt	(67)	(64)	(497)
Other financing activities, net	(178)	—	(564)
Net cash provided by financing activities	143,706	21,341	619,449
Increase (decrease) in cash and cash equivalents	85,983	(1,917)	273,644
Cash and cash equivalents:			
Beginning of period	187,661	39,904	—
End of period	$ 273,644	$ 37,987	$ 273,644
Supplemental disclosure of cash flow information:			
Cash paid for interest	$ 123	$ 65	$ 698
Supplemental schedule of noncash investing activities and financing activities:			
Short-term securities — available-for-sale received in connection with the private offering	$ —	$ —	$ 17,897
Issuance of common stock in satisfaction of warrant liabilities	$ 75,210	$ 391	$ 108,044
Tenant improvements paid by landlord	$ 3,692	$ —	$ 3,692
Issuance of common stock for building purchase	$ —	$ —	$ 750
Assumption of long-term debt for building purchase	$ —	$ —	$ 2,200
Issuance of common stock to acquire assets	$ —	$ —	$ 8,075
Assumption of liabilities to acquire assets	$ —	$ —	$ 2,124

See accompanying notes to condensed consolidated financial statements.

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

1. ORGANIZATION 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 the Company's 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 currently conducting an ongoing open label extension study following completion of its initial Phase IIb clinical trials. The Company is also focused on developing therapeutics for the treatment of infectious diseases, including its lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus for which the Company has historically received significant financial support from U.S. government research contracts.

The accompanying unaudited condensed consolidated financial statements reflect the accounts of Sarepta and its consolidated subsidiaries. The accompanying unaudited condensed consolidated balance sheet data as of December 31, 2012 was derived from audited financial statements not included in this report. The accompanying unaudited condensed consolidated financial statements were prepared in conformity with accounting principles generally accepted in the United States of America (GAAP) and the rules and regulations of the U.S. Securities and Exchange Commission (SEC) pertaining to interim financial statements. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. The accompanying unaudited condensed consolidated financial statements should be read in conjunction with the financial statements and the notes thereto included in the Company's annual report on Form 10-K for the year ended December 31, 2012. The results of operations for the interim periods presented are not necessarily indicative of the results to be expected for the full year.

Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others.

Since its inception in 1980, the Company has incurred losses of $534.4 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on change in warrant valuation partially offset by revenue generated from research contracts with and grants primarily from the U.S. Department of Defense (DoD). As of September 30, 2013, the Company has completed all of its contracts with the DoD except for the July 2010 contract for the development of therapeutics against the Marburg virus. The period of performance for the Company's August 2012 contract with the DoD related to the Marburg virus concluded in the third quarter of 2013. In November 2012, the Company entered into an agreement with the European Commission (EC) Health Innovation for development and study related activities for a DMD therapeutic. The Company has not generated any material revenue from product sales to date, and there can be no assurance that revenue from product sales will be achieved. Moreover, even if the Company does achieve revenue from product sales, the Company is likely to continue to incur operating losses in the near term.

As of September 30, 2013, the Company had $281.4 million of cash, cash equivalents and invested cash, comprised of $273.6 million of cash and cash equivalents and $7.8 million of restricted investments. The Company believes its cash, cash equivalents and invested cash is sufficient to fund the Company's current operational plan for the next twelve months. Should the Company's funding from the DoD cease or be delayed, the Company would likely curtail certain of its infectious disease research and development efforts unless additional funding was obtained. The Company is also likely to pursue additional cash resources through public or private financings, seeking additional government contracts, and from establishing collaborations or licensing its technology to other companies.

Estimates and Uncertainties

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. Actual results could differ from those estimates.

Commitments and Contingencies

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 employment, intellectual property, and effects from the use of therapeutics utilizing its technology, professional services or others. Although there are significant inherent uncertainties in connection with these legal matters, management believes the resolution of current matters will not have a material impact on the Company's financial position, results of operations or cash flows.

In February 2013, the Company issued two letters of credit totaling $7.3 million to a contract manufacturing vendor in connection with certain manufacturing agreements. The obligations secured by the letters of credit are fulfilled upon payment for certain minimum volume commitments that the Company expects to occur within the next twelve months. To meet the requirement of the letters of credit, the Company purchased $7.3 million in certificates of deposit with April 2014 maturity dates in February 2013. The Company has recorded this $7.3 million as restricted investments in the condensed consolidated balance sheet as of September 30, 2013.

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 upfront and development milestone payments. In April 2013, the Company recognized expense of $1.0 million relating to certain upfront payments required under the agreement within research and development in the condensed consolidated statement of operations.

In June 2013, the Company entered into a lease agreement for its Cambridge, Massachusetts location. 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 to meet the requirement. The initial term of the lease agreement is for seven years with an average base rent of approximately $2.4 million per year. At September 30, 2013, the Company recorded construction in progress related to the lease totaling $4.7 million in property and equipment in the condensed consolidated balance sheet of which $3.7 million was paid by the landlord as part of a tenant improvement allowance which is recorded in other long-term liabilities.

2. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares and dilutive common stock equivalent shares outstanding.

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
	(in thousands, except per share amounts)		(in thousands, except per share amounts)	
Net loss	$ (42,030)	$ (49,554)	$ (103,165)	$ (59,220)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of common shares outstanding for computing basic earnings per share	33,943	22,824	32,588	22,691
Dilutive effect of outstanding warrants and stock awards after application of the treasury stock method*	—	—	—	—
Weighted-average number of common shares outstanding for computing diluted earnings per share	33,943	22,824	32,588	22,691
Net loss per share — basic	$ (1.24)	$ (2.17)	$ (3.17)	$ (2.61)
Net loss per share — diluted	$ (1.24)	$ (2.17)	$ (3.17)	$ (2.61)

* Warrants, stock options, restricted stock units (RSUs) and stock appreciation rights (SARs) to purchase approximately 5,293,145 and 7,371,471 shares of common stock were excluded from the net loss per share calculation for the three and nine months ended September 30, 2013 and 2012, respectively, as their effect would have been anti-dilutive.

3. FAIR VALUE MEASUREMENTS

The Company measures at fair value certain financial assets and liabilities in accordance with a hierarchy of valuation techniques based on whether the inputs to those valuation techniques are observable or unobservable. Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect the Company's market assumptions. There are three levels of inputs that may be used to measure fair-value:

- 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 Company's assets and liabilities measured at fair value on a recurring basis consisted of the following as of the date indicated:

	Fair Value Measurement as of September 30, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Restricted investments, current	$ 7,250	$ 7,250	$ —	$ —
Restricted investments, noncurrent	557	557	—	—
Total assets	$ 7,807	$ 7,807	$ —	$ —

	Fair Value Measurement as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Restricted investments	$ —	$ —	$ —	$ —
Total assets	$ —	$ —	$ —	$ —

	Fair Value Measurement as of September 30, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	$ 35,994	$ —	$ —	$ 35,994
Total liabilities	$ 35,994	$ —	$ —	$ 35,994

	Fair Value Measurement as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	$ 65,193	$ —	$ —	$ 65,193
Total liabilities	$ 65,193	$ —	$ —	$ 65,193

* See Note 5 for additional information related to the determination of fair value of warrants and a reconciliation of changes in fair value.

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 and carrying amounts reported for long-term debt approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

4. ACCOUNTS RECEIVABLE

Accounts receivable are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily from the DoD and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. The accounts receivable balance included $4.2 million and $3.2 million of DoD receivables that were unbilled at September 30, 2013 and December 31, 2012, respectively.

5. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in January and August 2009 are classified as liabilities as opposed to equity because their settlement terms require settlement in registered shares, which is outside of the Company's control. These warrants are non-cash liabilities and the Company is not required to expend any cash to settle these liabilities. All other warrants issued by the Company were recorded as additional paid-in-capital and no further adjustments are made.

The outstanding warrants classified as liabilities are recorded at fair value on the condensed consolidated balance sheet and are adjusted to fair value at each financial reporting period, with changes in the fair value being recorded as "Loss on change in warrant valuation" in the condensed consolidated statement of operations and comprehensive income (loss). The fair value is 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. The following reflects the weighted-average assumptions for each of the periods indicated:

	September 30, 2013	December 31, 2012
Risk-free interest rate	0.1%	0.2%-0.3%
Expected dividend yield	0%	0%
Expected lives	0.8-0.9 years	1.1-1.6 years
Expected volatility (1)	67.8%-72.7%%	139.2%-164.1%
Shares underlying warrants classified as liabilities	959,283	3,127,618
Market value of stock at beginning of year	$ 25.80	$ 4.50
Market value of stock at end of period	$ 47.23	$ 25.80

(1) For the three and nine months ended September 30, 2013, expected volatility was estimated using a blend of calculated volatility of the Company's common stock over a historical period and implied volatility in exchange-traded options associated with the Company's common stock. Prior to January 1, 2013, expected volatility was estimated using calculated volatility of the Company's common stock over a historical period commensurate with the expected term of the option.

A reconciliation of the change in value of the Company's warrants recorded as liabilities for the three and nine months ended September 30, 2013 is as follows:

	Three months ended September 30, 2013 (in thousands)	Nine months ended September 30, 2013 (in thousands)
Balance at beginning of period	$ 79,116	$ 65,193
Increase in value of warrants	17,160	46,011
Reclassification to stockholders' equity upon exercise of warrants	(60,282)	(75,210)
Balance at end of period	$ 35,994	$ 35,994

For the nine months ended September 30, 2013, 2,168,335 warrants were exercised at a weighted average exercise price of $7.91, generating proceeds of $17.1 million. For the nine months ended September 30, 2012, 80,014 warrants were exercised at a weighted average exercise price of $4.08, generating proceeds of $0.3 million.

The following table summarizes the outstanding warrants at September 30, 2013.

Issue Date	Exercise Price	Outstanding Warrants at September 30, 2013	Expiration Date	Weighted Average Remaining Contractual Life (Years)	Exercisable Warrants
1/30/2009	$ 6.96	232,103	7/30/2014	0.8	232,103
8/25/2009	$ 10.68	727,180	8/31/2014	0.9	727,180
		959,283			959,283

6. EQUITY FINANCINGS

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

On July 3, 2013, the Company entered into a second ATM offering (the 2013 ATM) allowing the Company to sell, at its option, up to an aggregate of $125 million of shares of common stock at market prices. Through September 30, 2013, the Company has sold approximately 3.4 million shares under the 2013 ATM generating $123.0 million in net proceeds and has completed the sales of common stock available under the arrangement.

7. CONTRACT REVENUE

The Company recognizes revenue from U.S. and European Union (E.U.) government research contracts during the period in which the related expenditures are incurred and presents revenue and related expenses gross in the condensed consolidated financial statements. In the periods presented, substantially all of the revenue generated by the Company was derived from government research contracts.

The following table sets forth the revenue for each of the Company's contracts with the U.S. and E.U. governments and other revenue for the three and nine months ended September 30, 2013 and 2012.

	Three Months Ended September 30,		Nine months Ended September 30,	
	2013	2012	2013	2012
	(in thousands)		(in thousands)	
July 2010 Contract *(Ebola and Marburg IV)*	$ 2,444	$ 7,511	$ 7,134	$ 29,844
August 2012 Contract *(Intramuscular)*	514	50	2,759	50
November 2012 EU-SKIP-NMD Agreement *(DMD)*	536	—	599	—
July 2013 Children's National Medical Center	674	—	674	—
Other Agreements	—	13	427	99
Total	$ 4,168	$ 7,574	$ 11,593	$ 29,993

U.S. Government Contracts

As of September 30, 2013, the Company had completed all of its contracts with the DoD except for the Marburg portion of the July 2010 contract for the development of therapeutics against Ebola and Marburg viruses.

July 2010 Contract (Ebola and Marburg Intravenous administration)

On July 14, 2010, the Company was awarded a DoD contract managed by the Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office, a component of the Joint Program Executive Office for Chemical and Biological Defense, for the advanced development of the Company's hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003,
against the Ebola and Marburg viruses, respectively. In February 2012, the Company announced that it received permission from the U.S. Food and Drug Administration (FDA) to proceed with a single oligomer from AVI-6003, AVI-7288, as the lead product candidate against Marburg virus infection.

On August 2, 2012, the Company received a stop-work order related to the Ebola virus portion of the contract and, on October 2, 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The remaining Marburg portion of the contract is structured into four segments and has an aggregate remaining period of performance spanning approximately four years if the DoD exercises its options for all segments. Activities under the first segment began in July 2010 and include Phase I studies in healthy volunteers as well as preclinical studies.

After completion of the first segment, and each successive segment, the DoD has the option to proceed to the next segment. If the DoD exercises its options for segments II, III and IV, the Company's contract activities would include all clinical and licensure activities necessary to obtain FDA regulatory approval for the therapeutic candidate against the Marburg virus. The funding for segments II, III and IV of the Marburg virus portion of the contract is estimated to be approximately $84.4 million.

August 2012 Contract (Intramuscular administration)

On August 29, 2012, the Company was awarded a contract from the DoD, which is also managed by the JPM-TMT. The contract was awarded for approximately $3.9 million to evaluate the feasibility of an intramuscular (IM) route of administration using AVI-7288, the Company's candidate for treatment of Marburg virus. The period of performance of this contract concluded in the third quarter of 2013.

Other Agreements

For the nine month period ended September 30, 2013, Other Agreements includes $0.4 million in additional revenue from a former US government contract of the Company related to H1N1 influenza.

European Union SKIP-NMD Agreement

In November 2012, the Company entered into an agreement for a collaborative research project partially funded by the EC Health Innovation. The agreement provides for reimbursement of costs of approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic and is expected to last approximately three years.

During the nine months ended September 30, 2013, the Company received $1.3 million in advance payments and recognized $0.6 million of these payments as revenue. Deferred revenue related to the agreement as of September 30, 2013 was $0.7 million. The remaining balance of deferred revenue relates to the Company's sponsored research agreement with Charley's Fund from prior years.

Children's National Medical Center (CNMC) Agreement

In July 2013, the Company entered into an agreement totaling $1.3 million to provide drug product to CNMC to conduct research related to the Company's DMD program. During the three and nine months ended September 30, 2013, the Company has recognized $0.7 million as revenue under the agreement.

8. STOCK 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, RSUs and SARs. During the nine months ended September 30, 2013 there were no grants, exercises or cancelations of SARs.

Stock-based compensation costs are based on the fair value calculated utilizing the Black-Scholes-Merton option pricing model on the date of grant. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as compensation expense on a straight-line basis over the vesting period of the grants.

In June 2013, the Company's stockholders approved an additional 3.6 million shares of common stock available for grants under the Amended and Restated 2011 Equity Incentive Plan (the 2011 Plan) and stockholders approved the 2013 Employee Stock Purchase Plan (ESPP) with 250,000 shares of common stock available to be issued. As of September 30, 2013, 2,950,714 shares of common stock remain available for future grants under the 2011 Plan and 250,000 shares are available to be issued under the ESPP.

Stock Options

In general, stock options granted prior to December 31, 2010 vest over a three year period, with one-third of the underlying shares vesting on each anniversary of grant, and have a ten year term. Beginning in January 2011, stock options granted generally vest over a four year period, with one-fourth of the underlying shares vesting after one year and the remaining underlying shares vesting pro-ratably on a monthly basis thereafter, such that the underlying shares will be fully vested after four years.

In June 2013, the Company granted 459,500 stock options with performance-based vesting criteria. The performance criteria are based upon the achievement of certain clinical and regulatory milestones. As of September 30, 2013, the achievement of these performance criteria is not probable and accordingly the Company has not recognized any expense related to these options.

A summary of the Company's stock option activity with respect to the nine months ended September 30, 2013 follows:

Stock Options	Underlying Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term	Aggregate Intrinsic Value
Outstanding at December 31, 2012	2,522,522	$ 11.76		
Granted	2,063,396	34.86		
Exercised	(196,012)	8.51		
Canceled	(239,072)	11.51		
Outstanding at September 30, 2013	4,150,834	$ 23.37	8.71	$99,056,000
Vested at September 30, 2013 and expected to vest	3,395,249	$ 21.29	8.53	$ 88,063,000
Exercisable at September 30, 2013	891,157	$ 11.60	6.95	$ 31,748,000

The weighted-average fair value per share of stock-based awards granted to employees during the three months ended September 30, 2013 and 2012 was $26.92 and $7.09, respectively, and during the nine months ended September 30, 2013 and 2012 was $23.25 and $6.52, respectively. During the nine months ended September 30, 2013 and 2012, the total intrinsic value of stock options exercised was $5.1 million and $1.0 million, respectively. During the nine months ended September 30, 2013 and 2012, the total grant date fair value of stock options that vested was $3.0 million and $3.2 million, respectively.

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:

	Three and Nine months ended September 30,	
	2013	2012
Risk-free interest rate	0.7% - 1.4%	0.6% - 1.1%
Expected dividend yield	0%	0%
Expected lives	4.9-5.0 years	5.1-5.3 years
Expected volatility	80.0% - 88.9.%	79.7% - 94.8%

(1) For the three and nine months ended September 30, 2013, expected volatility was estimated using a blend of calculated volatility of the Company's common stock over a historical period and implied volatility in exchange-traded options associated with the Company's common stock. Prior to January 1, 2013, expected volatility was estimated using calculated volatility of the Company's common stock over a historical period commensurate with the expected term of the option.

Restricted Stock Awards (RSA)

In June 2013, the Company granted 6,000 shares of RSAs to members of its board of directors. These shares vest on the first anniversary of the grant and have a grant date fair value of $34.92 per share. 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 following table sets forth RSA activity for the period shown:

	Nine months ended September 30, 2013	
	Shares	Weighted Average Grant Date Fair Value per Share
Restricted Stock Awards, beginning of period	4,998	$ 10.08
Granted	6,000	34.92
Vested	(4,998)	10.08
Restricted Stock Awards, end of period	6,000	34.92

Restricted Stock Units (RSU)

In April 2012, the Company granted 32,377 shares of RSUs to employees in lieu of cash for a portion of the 2012 bonus. In addition, in August 2012, 7,500 RSUs were granted to an officer of the Company. The remaining RSUs at September 30, 2013 will vest by April 2014. The following table sets forth RSU activity for the period shown:

	Nine months ended September 30, 2013	
	Shares	Weighted Average Grant Date Fair Value per Share
Restricted Stock Units, beginning of period	38,260	$ 6.32
Granted	—	—
Vested	(24,858)	6.81
Canceled	(374)	5.40
Restricted Stock Units, end of period	13,028	$ 5.40

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 plan period. The initial 26-month award period will end on August 31, 2015. Each subsequent offering period will begin on March 1 or September 1.

For the three and nine months ended September 30, 2013, the fair value of stock purchase rights ranges from $16.12 to $24.65 per share on 59,563 shares estimated to be purchased during the initial award period. The fair value was estimated using the Black-Scholes-Merton option-pricing model. The Company used a weighted-average stock-price volatility ranging from 84% to 98%, expected option life assumption from 0.7 to 2.2 years and a risk-free interest rate from 0.1% to 0.4%. The Company recorded $0.3 million of stock-based compensation expense for the three and nine months ended September 30, 2013 related to the ESPP.

Stock-based Compensation Expense

A summary of the stock-based compensation expense, including stock options, RSAs, RSUs, SARs, and ESPP rights recognized in the condensed consolidated statements of operations and comprehensive income (loss) is as follows:

	Three Months Ended		Nine months Ended	
	September 30, 2013	September 30, 2012	September 30, 2013	September 30, 2012
	(in thousands)		(in thousands)	
Research and development	$ 1,155	$ 271	$ 2,409	$ 783
General and administrative	2,332	421	5,067	1,057
Total	$ 3,487	$ 692	$ 7,476	$ 1,840

As of September 30, 2013, there was $49.6 million of unrecognized compensation cost related to non-vested share-based compensation arrangements outstanding including stock options, RSAs, RSUs, SARs and ESPP rights. These costs are expected to be recognized over a weighted-average period of 3.2 years.

9. INCOME TAXES

At December 31, 2012, the Company had net deferred tax assets of approximately $114.1 million. The net deferred tax assets are primarily composed of U.S. federal and state tax net operating loss (NOL) carryforwards, U.S. federal and state research and development credit carryforwards and share-based compensation expense. 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 its NOL and research and development credit carryforwards to offset future taxable income based on ownership changes and the value of the Company's stock.

10. RESTRUCTURING

In November 2012, the Company notified 21 employees based in Bothell, Washington that they would be terminated as part of the relocation of the Company's corporate headquarters to Cambridge, Massachusetts. The employees were given various incentives to remain through a transition period which is expected to be completed in 2013. For the nine months ended September 30, 2013, the Company recorded restructuring charges of $0.4 million to research and development expense and $0.3 million to general and administrative expense. All transition costs are expected to be paid in 2013.

Changes in the liability and the balance related to the restructuring plan are as follows:

	Nine months ending September 30, 2013
	(in thousands)
Balance at December 31, 2012	$ 185
Restructuring charges	726
Payments	(821)
Balance at September 30, 2013	$ 90

11. RECENT ACCOUNTING PRONOUNCEMENTS

In February 2013, the Financial Accounting Standards Board (FASB) issued new guidance which requires disclosure of significant amounts reclassified out of accumulated other comprehensive income by component and their corresponding effect on the respective line items of net income. This guidance was adopted by the Company in fiscal year 2013. The adoption of this guidance did not have an impact on the Company's unaudited condensed consolidated financial statements.

In July 2013, the FASB issued new guidance which amends the guidance related to the presentation of unrecognized tax benefits and allows for the reduction of a deferred tax asset for a NOL carryforward whenever the NOL or tax credit carryforward would be available to reduce the additional taxable income or tax due if the tax position is disallowed. The new guidance is effective for annual and interim periods for fiscal years beginning after December 15, 2013, and early adoption is permitted. Since the guidance relates only to the presentation of unrecognized tax benefits, the Company does not expect its adoption in January 2014 will have a material effect on its financial position, results of operations or cash flows.

12. SUBSEQUENT EVENTS

The Company evaluated events and transactions after the date of the balance sheet data but prior to the issuance of the financial statements for potential recognition or disclosures in its financial statements. The Company did not identify any material subsequent events requiring adjustment or disclosure.

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, 2012 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 development and clinical benefits of our product candidates;*
- *the results of our research and development efforts and the efficacy of our PMO-based chemistries and other RNA-based technology;*
- *our expectations regarding our ability to become a leading developer and marketer of RNA-based therapeutics;*
- *the efficacy, potency and utility of our product candidates in the treatment of rare and infectious diseases, and their potential to treat a broad number of human diseases;*
- *our expectations regarding the results of preclinical and clinical testing of our product candidates;*
- *our expectations regarding the timing for initiating a confirmatory clinical study and for filing a new drug application for eteplirsen;*
- *our expectations regarding the timing, completion and receipt of results from our ongoing development programs;*
- *the timing of and requirements the Company must comply with to receive any required approvals from the U.S. Food and Drug Administration (FDA) or other regulatory approvals for our products;*
- *the effect of regulation by the FDA and other agencies on the Company and development of our product candidates;*
- *our expectations regarding the markets for our products;*
- *acceptance of our products, if introduced, in the marketplace;*
- *the impact of competitive products, product development, 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 to file additional patent applications to enhance and protect our existing intellectual property portfolio;*
- *our ability to invalidate some or all of the claims covered by patents issued to competitors;*

- *our estimates regarding our future revenues, research and development expenses, other expenses, payments to third parties and changes in staffing levels;*
- *our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and statements about our future capital needs;*
- *our ability to increase the scale of our manufacturing to provide our product to patients in larger scale clinical trials or in potential commercial quantities;*
- *our ability to operate our business without infringing the intellectual property rights of others;*
- *our expectations about funding from government and other sources; 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 Duchenne muscular dystrophy (DMD) drug candidates, including our lead product candidate, eteplirsen. We are also focused on developing therapeutics for the treatment of infectious diseases, including our lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus. By building our infectious disease programs which are primarily funded and supported by the U.S. Department of Defense (DoD), and leveraging our highly-differentiated, proprietary technology platforms, we are seeking to further develop our research and development competencies and identify additional product candidates.

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. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe unmet medical need. Last year, we completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, we initiated an open label extension study with the same participants from the original Phase IIb placebo controlled trial. We are working with the FDA to initiate a confirmatory clinical study and submit an NDA filing for eteplirsen although the timing for these is unclear at this time.

We are also leveraging the capabilities of our RNA-based technology platforms to develop therapeutics for the treatment of infectious diseases. The DoD has provided significant financial support in the past for the development of therapeutics against Ebola, Marburg, Dengue and influenza viruses. We have attracted DoD's support based in part on our ability to rapidly respond to pathogenic threats by quickly identifying, manufacturing and evaluating novel therapeutic candidates.

The basis for our novel RNA-based therapeutics is our phosphorodiamidate-linked morpholino oligomer, or 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.

On July 12, 2012, our common stock began trading on The NASDAQ Global Market on a split-adjusted basis following a one-for-six reverse stock split that was effective on July 11, 2012. Unless otherwise noted, all share amounts, share prices and exercise prices included throughout this report give effect to the July 2012 one-for-six reverse stock split.

Since our inception in 1980, we have incurred losses of $534.4 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on changes in warrant valuation partially offset by revenue generated from research contracts with and grants primarily from the DoD. As of September 30, 2013, we have completed all of our contracts with the DoD except for the July 2010 contract for the development of therapeutics against the Marburg virus. The period of performance for our August 2012 contract with the DoD concluded in the third quarter of 2013. In November 2012 we also entered into an agreement with the EC Health Innovation for development and study related activities for a DMD therapeutic for which minimal revenues have been earned to date. We have not generated any material revenue from product sales to date, and there can be no assurance that revenues from product sales will be achieved. Moreover, 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, 2013, we had $281.4 million of cash, cash equivalents and invested cash, comprised of $273.6 million of cash and cash equivalents and $7.8 million of restricted investments, which we believe, taking into consideration our current stock price and outstanding warrants, is sufficient to fund our current operational plan for the next twelve months. Should our funding from the DoD cease or be delayed, we would likely curtail certain infectious disease research and development efforts unless additional funding was obtained. We are also likely to pursue additional cash resources through public or private financings, seeking additional government contracts, and by establishing collaborations or licensing our technology to other companies.

We were originally incorporated in the State of Oregon on July 22, 1980 and on June 6, 2013, we reincorporated in Delaware. Our principal executive offices are located at 215 First Street, Suite 7, Cambridge, MA 02142 and our telephone number is (857) 242-3700. Our common stock trades on The NASDAQ Global Market under the symbol “SRPT.”

Government Contracts

We recognize revenue from government research contracts during the period in which the related expenditures are incurred and present these revenues and related expenses gross in the condensed consolidated financial statements. In the periods presented, substantially all of the revenues generated by us were derived from research contracts with the DoD. As of September 30, 2013, we had completed all of our contracts with the DoD except for the Marburg portion of the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses.

The following table sets forth the revenue from each of our contracts with the U.S. and E.U. governments and other revenue for the three and nine months ended September 30, 2013 and 2012.

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
	(in thousands)		(in thousands)	
July 2010 Contract (*Ebola and Marburg IV*)	$ 2,444	$7,511	$ 7,134	$ 29,844
August 2012 Contract (*Intramuscular*)	514	50	2,759	50
November 2012 SKIP-NMD Agreement (*DMD*)	536	—	599	—
July 2013 Children’s National Medical Center	674	—	674	—
Other Agreements	—	13	427	99
Total	$4,168	$7,574	$11,593	$29,993

July 2010 Contract (Ebola and Marburg Intravenous administration)

On July 14, 2010, we were awarded the DoD contract managed by the JPM-TMT Project Management Office for the advanced development of our hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003, against the 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-6003, AVI-7288, as the lead product candidate against Marburg virus infection.

On August 2, 2012, we received a stop-work order related to the Ebola virus portion of the contract and, on October 2, 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The remaining Marburg portion of the contract is structured into four segments and has an aggregate remaining period of performance spanning approximately four years if DoD exercises its options for all segments. Activities under the first segment began in July 2010 and include Phase I studies in healthy volunteers as well as preclinical studies.

After completion of the first segment, and each successive segment, DoD has the option to proceed to the next segment. If DoD exercises its options for segments II, III and IV, our contract activities would include all clinical and licensure activities necessary to obtain FDA regulatory approval for the therapeutic candidate against the Marburg virus. The funding for segments II, III and IV of the Marburg virus portion of the contract is estimated to be approximately $84.4 million.

August 2012 Contract (Intramuscular administration)

On August 29, 2012, we were awarded a contract from the DoD, which is also being managed by the JPM-TMT. The contract was awarded for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, our candidate for treatment of Marburg virus. The period of performance for this contract concluded in the third quarter of 2013.

European Union SKIP-NMD Agreement (DMD)

In November 2012, we entered into an agreement for a collaborative research project partially funded by the EC Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic and is expected to last approximately three years.

During the nine months ended September 30, 2013, we received $1.3 million in advance payments and recognized $0.6 million of these payments as revenue. Deferred revenue related to the agreement as of September 30, 2013 was $0.7 million. The remaining balance of deferred revenue relates to our sponsored research agreement with Charley's Fund.

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

In July 2013, we entered into an agreement totaling $1.3 million to provide drug product to CNMC to conduct research related to the Company's DMD program. During the three and nine months ended September 30, 2013, the Company has recognized $0.7 million as revenue under the agreement.

Other Agreements

For the nine month period ended September 30, 2013, Other Agreements includes $0.4 million in additional revenue from our former U.S. government contract related to H1N1 influenza.

Key Financial Metrics

Revenue

Government Research Contract and Grant Revenue. Substantially all of our revenue is generated from U.S. government research contracts and grants. See Note 7 of the Notes to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this Quarterly Report on Form 10-Q. We recognize revenue from government research contracts and grants during the period in which the related expenses are incurred and present such revenue and related expenses gross in the condensed consolidated financial statements. Government contract revenue is highly dependent on the timing of various activities performed by us and our third party vendors. Changes in the timing of activities performed in support of these contracts have, and may in the future, result in unexpected fluctuations in our revenue from period to period. We expect that future revenue generated under our government contracts will continue to be variable as a result of these factors.

License Arrangements. Our license arrangements may consist of non-refundable upfront license fees, data transfer fees, research reimbursement payments, exclusive licensed rights to patented or patent pending compounds, technology access fees, various performance or sales milestones and future product royalty payments. Some of these arrangements are multiple element arrangements.

We defer recognition of non-refundable upfront 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, then such up-front fees are deferred and recognized over the period of continuing involvement. As of September 30, 2013, we had deferred revenue of $4.0 million, which represents up-front fees which we will recognize as revenue as we satisfy the outstanding performance obligations.

Expenses

Research and Development. Research and development expense consists of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, and clinical trial and manufacturing costs.

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

The amount and timing of future research and development expense will depend in part on our ability to obtain U.S. government awards to fund the advanced development of our infectious disease therapeutic candidates. Without such funding, we would likely significantly reduce our spending in these areas. Future research and development expenses may also increase as our internal projects, such as eteplirsen for DMD, enter later stage clinical development. Our research and development programs are in ongoing open label extension studies of Phase IIb clinical trials or in earlier stages and may not result in any approved products. 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 we have 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 and 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 and sale of any product candidate. The timeframe 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 expense consists principally of salaries, benefits, stock-based compensation expense, and related costs for personnel in our executive, finance, legal, information technology, business development, investor relations and human resource 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 on our cash and cash equivalents, interest expense, and rental income. Our cash equivalents consist of money market investments. Interest expense includes interest on our mortgage loan related to the Corvallis property, the substantial portion of which we leased in November 2011. Rental income is from subleasing excess space in some of our facilities.

Loss on Change in Warrant Liability. Warrants issued in connection with our January and August 2009 financings are classified as liabilities as opposed to equity due to their settlement terms. These warrants are non-cash liabilities; we are not required to expend any cash to settle these liabilities. The fair market value of these warrants was recorded on the balance sheet at issuance and the warrants are marked to market each financial reporting period, with changes in the fair value recorded as a gain or loss in our statement of operations and comprehensive income (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 market valuation primarily due to changes in our stock price. For more information, see Note 5 of the Notes to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this Quarterly Report on Form 10-Q.

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 condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States, or 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 that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our 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;
- stock-based compensation; and
- accounting for and valuation of warrants classified as liabilities.

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, 2012 filed with the Securities and Exchange Commission, or SEC, on March 15, 2013.

Results of Operations for the Three and Nine months Ended September 30, 2013 and 2012

The following table sets forth selected consolidated statements of operations and comprehensive income (loss) data for each of the periods indicated:

	Three months ended September 30,		% Change	Nine months ended September 30,		% Change
	2013	2012		2013	2012	
	(in thousands, except per share amounts)			(in thousands, except per share amounts)		
Revenue	$ 4,168	$ 7,574	(45)%	$ 11,593	$ 29,993	(61)%
Operating expenses:						
Research and development	21,087	10,914	93%	47,833	39,568	21%
General and administrative	8,014	3,565	125%	21,195	9,761	117%
Operating loss	(24,933)	(6,905)	261%	(57,435)	(19,336)	197%
Other income (loss):						
Interest income and other, net	63	67	(6)%	281	270	4%
Loss on change in warrant liability	(17,160)	(42,716)	(60)%	(46,011)	(40,154)	15%
Net loss	$ (42,030)	$(49,554)	(15)%	$(103,165)	$(59,220)	74%
Basic net loss per share	$ (1.24)	$ (2.17)		$ (3.17)	$ (2.61)	
Diluted net loss per share	$ (1.24)	$ (2.17)		$ (3.17)	$ (2.61)	

Revenue

Revenue for the three months ended September 30, 2013 decreased by $3.4 million, or 45%, compared to the three months ended September 30, 2012. The decrease was primarily caused by a $2.8 million decrease in revenue from the Ebola portion of the DoD contract due to the August 2012 stop-work order and the subsequent termination for convenience in October 2012. Additionally, there was a decrease of $2.3 million in revenue associated with the Marburg portion of the DoD contract. The decrease was partially offset by a $1.7 million increase in revenue earned on the IM Marburg government research contract, the EU-SKIP grant agreement and the CNMC agreement.

Revenue for the nine months ended September 30, 2013 decreased by $18.4 million, or 61%, compared to the nine months ended September 30, 2012. The decrease was primarily due to a $12.4 million decrease in revenue from the Ebola portion of the DoD contract as a result of the August 2012 stop-work order and the subsequent termination for convenience in October 2012. Additionally, there was a decrease of $10.3 million in revenue associated with the Marburg portion of the DoD contract. The decrease was partially offset by a $4.3 million increase in revenue earned on the IM Marburg government research contract, the EU-SKIP grant agreement, the CNMC agreement and other revenue related to prior and completed government contracts.

Research and Development Expenses

Research and development expenses for the three months ended September 30, 2013 increased by $10.2 million, or 93%, compared to the three months ended September 30, 2012. The increase was primarily due to a $6.0 million increase in our DMD program costs due to the timing of manufacturing and clinical activities, an increase of $5.8 million in costs of proprietary research, and an increase of $1.7 million in personnel related costs which includes $0.9 million in increased stock-based compensation expense. The increase was partially offset by a $2.1 million decrease in costs under the Ebola portion of the DoD contract due to the August 2012 stop-work order and the subsequent termination for convenience in October 2012, and a decrease of $1.7 million in costs under the Marburg portion of the DoD contract.

Research and development expenses for the nine months ended September 30, 2013 increased by $8.3 million, or 21%, compared to the nine months ended September 30, 2012. The increase was primarily due to a $12.2 million increase in costs of proprietary research which includes $1.0 million in license fees related to an agreement for exclusive rights on certain intellectual property, a $6.6 million increase in our DMD program costs, a $1.9 million increase in costs incurred on the IM government research contract, and an increase of $3.9 million in personnel related costs which includes $1.6 million in increased stock-based compensation expense. The increase in costs was offset by an $8.6 million decrease in costs under the Ebola portion of the DoD contract as a result of the August 2012 stop-work order and the subsequent termination for convenience in October 2012 and a decrease of $8.0 million in costs on the Marburg portion of the DoD contract.

General and Administrative Expenses

General and administrative expenses for the three months ended September 30, 2013 increased by $4.4 million, or 125%, compared to the three months ended September 30, 2012. The increase in general and administrative expenses is primarily due to a $2.8 million increase in personnel costs including $1.9 million in stock-based compensation from additional headcount, $0.6 million of additional cost associated with facilities and $1.0 million of additional professional services and other costs.

General and administrative expenses for the nine months ended September 30, 2013 increased by $11.4 million, or 117%, compared to the nine months ended September 30, 2012. The increase in general and administrative expenses is primarily due to a $7.8 million increase in personnel costs including $4.0 million in stock-based compensation from additional headcount, $0.8 million of additional cost associated with facilities, $1.5 million of additional professional services and $1.4 million of other costs.

Interest Income and Other, Net

Interest income and other, net, for the three and nine months ended September 30, 2013 remained consistent compared to the three and nine months ended September 30, 2012.

Loss on Change in Warrant Liability

The change in fair value of our warrant liability for the three and nine months ended September 30, 2013 compared to the three and nine months ended September 30, 2012 was primarily attributable to the change in our stock price. See Note 5 to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this report.

Net Loss

Net loss for the three months ended September 30, 2013 was $42.0 million, compared to net loss of $49.6 million for the three months ended September 30, 2012, a decrease of $7.6 million. The decrease in net loss was primarily due to increased operating losses and offset by the decrease in loss in the change in our warrant liability.

Net loss for the nine months ended September 30, 2013 was $103.2 million, compared to net loss of $59.2 million for the nine months ended September 30, 2012. The increased net loss was primarily due to an increase in operating loss of $38.1 million and a $5.9 million increase in non-operating expense due to the increase in the fair market value of our outstanding warrants. The fair market value of our outstanding warrants is a non-cash expense which is highly impacted by the change in the value of our stock.

Liquidity and Capital Resources

Our principal sources of liquidity are revenue from government research contracts and grants, and equity transactions. As of September 30, 2013, we had $281.4 million of cash, cash equivalents and invested cash, comprised of $273.6 million of cash and cash equivalents and $7.8 million of restricted investments, compared to $187.7 million of cash and cash equivalents at December 31, 2012. The increase during the nine month period ended September 30, 2013 is due primarily to cash provided by sale of approximately 3.4 million shares of our common stock under our ATM financing agreements which generated $125.1 million in net proceeds. In addition, we issued approximately 2.4 million shares of our common stock from the exercise of warrants and options generating $18.9 million in cash. The increase was offset by funds used to operate our business and for investing activities. We believe our available cash, cash equivalents and invested cash is sufficient to fund our current operational plan for the next twelve months.

Our primary source of revenue is from development of product candidates pursuant to our contracts with the U.S. and E.U. governments. Government funding is subject to the U.S. government's appropriations process and the U.S. government has the right under our contracts with them to terminate such contracts for convenience as was done regarding the Ebola portion of the 2010 Ebola and Marburg contract. If DoD funding is not received or is delayed, we would likely curtail certain of our infectious disease research and development efforts unless additional funding was obtained. Currently, we do not generate any revenue from the commercial sale of our pharmaceutical product candidates.

Our future expenditures and capital requirements depend on numerous factors, most of which are difficult to project beyond the short term. These requirements include the progress of our research and development programs and our pre-clinical and clinical trials, our ability to meet the requirements of our DoD research projects, the time and costs involved in obtaining regulatory approvals, the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, competing technological and market developments, our ability to establish collaborative arrangements and the terms of any such arrangements, and the costs associated with manufacturing and commercialization of our products.

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 assure you 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 it, it 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.

Historical Trends

	Nine months ended September 30,	
	2013	2012
	(in thousands)	
Cash provided by (used in):		
Operating activities	$ (46,873)	$ (22,536)
Investing activities	(10,850)	(722)
Financing activities	143,706	21,341
Increase (Decrease) in cash and cash equivalents	$ 85,983	$ (1,917)

Operating Activities. The increase in the amount of cash used in operating activities of $24.3 million for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 was primarily due to an increase in operating loss of $38.1 million driven by lower government contract revenue and higher research and development costs and higher general and administrative costs. In addition to the increase in operating loss, there was a favorable change in operating assets and liabilities of $7.9 million, as well as a $5.6 million increase in stock-based compensation costs, which is a non-cash adjustment to net loss.

Investing Activities. The increase in the amount of cash used in investing activities of $10.1 million for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 was due to the purchase of $7.3 million of investments in February 2013 to secure two letters of credit issued in connection with certain manufacturing contracts, and due to the purchase of a $0.6 million investment to secure a letter of credit for a security deposit relating to our Cambridge lease. Also there was an increase in cash used to fund patent costs of $0.7 million and an increase in capital expenditures of $1.7 million in the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012.

Financing Activities. The increase in the amount of cash from financing activities of $122.4 million for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 was primarily due to the sale of approximately 3.4 million shares of common stock under the 2013 ATM sales agreement which generated $123.0 million in net proceeds, in addition to the sale of approximately 87,000 shares of common stock under the 2012 ATM sales agreement in January 2013 which generated $2.1 million in net proceeds and fully exhausted the sales of our stock available under the 2012 ATM sales agreement. We also received $17.1 million in net proceeds from warrant exercises and $1.7 million from stock option exercises during the nine months ended September 30, 2013 for which we issued approximately 2.4 million shares of additional common stock. During the nine months ended September 30, 2012 we received $21.4 million in net proceeds from sale of common shares and the exercise of warrants and options.

Contractual Obligations

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, 2013:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
	(in thousands)				
Long-term debt	$ 1,690	$ 91	$ 198	$ 219	$ 1,182
Operating leases	29,260	3,791	7,773	8,029	9,667
Purchase obligations (1)	169,648	42,753	87,680	35,650	3,565
Total	$ 200,598	$46,635	$95,651	$ 43,898	$ 14,414

(1) 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.

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

See Note 11 to the Notes to the Unaudited Condensed Consolidated Financial Statements contained in Part I, Item 1 of this report.

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

At September 30, 2013, we had $281.4 million of cash, cash equivalents and invested cash, comprised of $273.6 million of cash and cash equivalents and $7.8 million of restricted investments, compared to $187.7 million of cash and cash equivalents at December 31, 2012. We do not enter into investments for trading or speculative purposes; our cash equivalents are invested in money market accounts. We believe that we do not have any material exposure to changes in the fair value of these assets in the near term due to the short term nature of our cash and cash equivalents. A 0.1% decline in interest rates, occurring January 1, 2013 and sustained throughout the period ended September 30, 2013, would have been inconsequential.

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, under the supervision and with the participation of our management, including (1) our chief executive officer and (2) our chief financial officer, of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act. 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 Securities and Exchange Commission, or 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) 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, 2013, our disclosure controls and procedures were effective.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting during the quarter ended September 30, 2013 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings.

As of the date of this report, we are not a party to any material legal proceedings with respect to us, our subsidiaries, or any of our material properties. In the normal course of business, we may from time to time be named as a party to various legal claims, actions and complaints, including matters involving employment, intellectual property, effects from the use of drugs utilizing our technology, or others. Although there are significant inherent uncertainties in connection with these legal matters, management believes the resolution of current matters will not have a material impact on the Company's financial position, results of operations or cash flows.

Item 1A. Risk Factors.

Set forth below and elsewhere in this report and in other documents we file with the 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

Our product candidates are at an early stage of development, and it is possible that none of our product candidates will ever become commercial products.

Our product candidates are in relatively early stages of development. These product candidates will require significant further development, financial resources and personnel to obtain regulatory approval and develop into commercially viable products, if at all. Currently, eteplirsen in DMD, AVI-7288 in Marburg and AVI-7100 in influenza are in active clinical development. AVI 7537 in Ebola is no longer in clinical development as a result of the October 2012 notice we received from the DoD, terminating the program for the development of AVI-7537 for the convenience of the government due to funding constraints. The rest of our product candidates are in preclinical development. We expect that much of our effort and many of our expenditures over the next several years will be devoted to development 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. With current resources, we may be restricted or delayed in our ability to develop these and other clinical and preclinical product candidates.

Our ability to commercialize any of our product candidates, including eteplirsen, depends on first receiving required regulatory approvals. It is possible that our product candidates, including eteplirsen, may never receive regulatory approval, including any accelerated approval by the FDA under Subpart H — Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses, or any other designations that will expedite the review or approval process for various reasons including: any failure to meet the applicable regulatory requirements to obtain regulatory approval for any of our product candidates including any failure to demonstrate the safety and effectiveness for any of our product candidates, lack of funding, changes in the regulatory landscape, new scientific developments, including the results for clinical trials of competitor drugs, and the FDA's interpretation and analysis of such developments in connection with our product candidates, manufacturing or other reasons. If we are unable to obtain regulatory approval for any of our current product candidates, it could delay or eliminate any potential product commercialization and product revenue for our Company.

Even if a product candidate receives regulatory approval, the resulting product may not gain market acceptance among physicians, patients, healthcare payers and the medical community. Assuming that any of our product candidates receives the required regulatory approvals, commercial success will depend on a number of factors, including:

- establishment and demonstration 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 governmental payers such as the Medicare and Medicaid programs, managed care organizations, and private health insurers;
- the product's potential advantage over alternative treatment methods;
- whether the product can be produced in commercial quantities and at acceptable costs;

- marketing and distribution support for the product; and
- any exclusivities applicable to the product.

If we are unable to develop and commercialize any of our product candidates, if development is delayed or if sales revenue from any product candidate that receives marketing approval is insufficient, we may never reach sustained profitability.

We have been granted orphan disease status for certain of our product candidates, but there can be no assurance 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 status under the Orphan Drug Act by the FDA for: two of our product candidates in DMD (including eteplirsen), AVI-6002 and AVI-7537 for the treatment of Ebola virus and AVI-6003 and AVI-7288 for the treatment of Marburg virus. Generally, product candidates granted orphan status are provided with seven years of marketing exclusivity by the FDA upon New Drug Application (NDA) approval, meaning the FDA will generally not approve applications for product candidates that contain the same active ingredient and are labeled for the same orphan indication. Even if we are the first to obtain marketing exclusivity through an approval of an orphan product in the United States, there are limited circumstances under which a later product from a competitor 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 drug medicinal product designations in the European Union for our lead drug candidate, eteplirsen, and AVI-5038 for the treatment of DMD. 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. Pediatric product candidates may be eligible for an additional two years of marketing exclusivity. Although we may have drug candidates that have or may obtain orphan drug exclusivity in Europe, the orphan designation and associated exclusivity period may be modified for several reasons, including the designation criteria have significantly changed since market authorization of the orphan product, (e.g. product profitability exceeds the criteria for orphan drug designation), there are production or supply problems with 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 have been granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the E.U., our business and results of operations could be materially adversely affected. While orphan drug status for any of our products would provide market exclusivity in the U.S. and Europe, 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 designation. Moreover, we cannot guarantee that another company will not receive approval before we do of an orphan drug application in the U.S. or the E.U. for a product candidate that has the same active ingredient or is a similar medicinal product, respectively, for the same indication as any of our drug candidates for which we plan to file for orphan 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 U.S. or the E.U. 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.

If we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates, our ability to generate revenue will be materially impaired and our business will not be successful.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by state authorities and the FDA in the United States and other regulatory authorities in other countries, with regulations differing from country to country. Marketing of our product candidates in the United States or foreign countries is not permitted until we obtain the required approvals from the FDA or other applicable foreign regulatory authorities. Obtaining marketing approval is generally a lengthy, expensive and uncertain process in the U.S. and other countries and approval is not assured for any of our product candidates.

Further, the FDA and other foreign regulatory agencies have substantial discretion in the approval process, and the determination of when or whether regulatory approval, of any type, will be granted for any product candidate we develop. In this regard, even if we

believe data collected from clinical trials of our product candidates are promising and our chemistry, manufacturing and controls (CMC) and related manufacturing processes are satisfactory, the FDA or foreign authorities may disagree with our interpretations and determine such data is not sufficient to accept our application or support approval. Furthermore, the FDA or other foreign regulatory agencies may approve a product candidate for fewer indications than requested or may grant approval subject to the performance of post-approval or confirmatory studies for a product candidate. Similarly, the FDA or other foreign regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

In addition, changes in (i) regulatory requirements, (ii) FDA interpretations of scientific developments in diseases targeted by us or our competitors or data and information we submit to the FDA about our product candidates and (iii) FDA guidance and requirements for approval may occur and we may need to amend clinical trial protocols or our approval strategies, including the timing of expected Company filings with the FDA, to reflect or address these changes. These changes or amendments may require us to resubmit our clinical trial protocols to institutional review boards (IRBs) or the FDA for review, which may impact the costs, timing or successful completion of a clinical trial. A therapeutic commercial product utilizing our RNA-based technologies and the manufacturing techniques necessary to produce them at commercial scale have never been approved or validated by any regulatory authority and the FDA may require the Company to make or develop changes in its protocols that will take time, sometimes not estimable, to develop. Changes in the approval process for our product candidates, including those described above, may require additional studies or require the Company to address additional issues or requests that were not originally planned, budgeted for or expected by the Company. Other factors may also impact our ability to obtain or impact the timing of approval for our product candidates, affect the receptiveness of regulators to our compounds, protocols or otherwise impact the regulatory process for our drug candidates including regulatory or other setbacks faced by third parties developing similar compounds or developing drug candidates targeting the same, similar or related diseases as those targeted by our drug candidates. For example, in our most recent meeting with the FDA, based on recent developments in natural history studies and other data from clinical trials for investigational drugs developed by other companies, the FDA indicated it has considerable doubt about the use of dystrophin as a biomarker and questioned the efficacy support provided by the 6MWT in our ongoing open label study. Our exon-skipping therapy uses antisense oligonucleotides and, to date, only one antisense oligonucleotide has been approved by the FDA for systemic use and no product using antisense oligonucleotides for systemic use has been approved for sale in the European Union. We cannot be certain that our technology will meet applicable safety and efficacy standards or that we will be able to comply with all the requirements of regulatory authorities. Due to these factors, among others, our current product candidates or any of our other future 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 any of our product candidates.

We continue to work with the FDA in our pursuit to obtain FDA approval of eteplirsen. The Company believes that the new issues and concerns raised by the FDA at its last meeting with the Company may result in delays in commencing the Company's confirmatory clinical study as well as its filing of an NDA for eteplirsen. The Company does not currently have enough information at this time to determine the length of such delays. Furthermore, there can be no assurance that any submission or application will be accepted by the FDA (*e.g.*, refusal to file) or that any expedited or regular development, review or approval will be granted on a timely basis, or at all. The FDA or other foreign authorities could also request additional information or meetings with us or require us to conduct further studies or CMC-related work (*e.g.*, a complete response letter) prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for eteplirsen or any of our other product candidates would result in a longer time period for commercialization of such product candidate, could potentially increase the cost of development of such product candidate, could have a material adverse effect on our financial condition and could harm our competitive position in the marketplace.

Additionally, even if we receive regulatory approval for our product candidates, we will be subject to ongoing FDA obligations and oversight, including adverse event reporting requirements, marketing restrictions and, potentially, other post-marketing obligations such as confirmatory studies, all of which may result in significant expense and limit our ability to commercialize any such products. The FDA's policies may also change and additional government regulations may be enacted that could further restrict or regulate post-approval activities. We cannot predict the likelihood, nature or extent of adverse government regulation that may arise from future legislation or administrative action, either in the United States, or abroad. If we are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties, we may not be permitted to market our products and our business could suffer.

Any delay in, or failure to, receive or maintain regulatory approval for any of our product candidates could harm our business and prevent us from ever generating meaningful revenues or achieving profitability. We will also need to obtain regulatory approval from regulatory authorities in foreign countries to market our product candidates in those countries. We have not submitted an application for regulatory approval to market our product candidates in any foreign jurisdiction. Approval by one regulatory authority does not ensure approval by regulatory authorities in other jurisdictions. If we fail to obtain approvals from foreign jurisdictions, the geographic market for our product candidates would be limited.

Our preclinical and clinical trials may fail to demonstrate acceptable levels of safety and efficacy 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 or efficacy to obtain regulatory approvals.

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 at weeks 24 and 48 respectively, and results reported for weeks 62, 74, 84 and 96 supported stabilization of disease progression, we cannot assure you that data from the ongoing open label extension study will be sufficient for regulatory approval or will continue to be positive through the remaining study period. If these extension data are not sufficient to demonstrate safety and efficacy to regulators, do not continue to demonstrate safety and efficacy through the remainder of Study 202, or are insufficient to identify a consistently effective dose, we expect we will need to engage in discussions with regulatory authorities about the design and subsequent execution of any further studies that may be required. Regulatory authorities might require more extensive information or preclinical or clinical trials than anticipated. Such clinical trials might include additional open label "extension studies" for all participants, who have previously received eteplirsen, as well as other participants (*e.g.*, non-ambulatory participants), additional placebo-controlled "pivotal" study or studies, or additional trials before conducting a pivotal trial or trials of the product candidate. Any additional requirements for regulatory approval would increase our costs and delay submissions, studies and commercialization of eteplirsen. Even if we conform to any guidance regulatory authorities provide, it does not guarantee receipt of marketing approval, even if we believe our preclinical and clinical trials are successful.

Furthermore, success in preclinical and early clinical trials does not ensure that the ongoing Study 202 and later larger-scale trials will be successful nor does it predict final results. Acceptable results in early trials may not be reproduced in the remainder of the Study 202 extension study or later trials. For example, pivotal trials for eteplirsen will likely involve a larger number of patients to achieve statistical significance, will be expensive and will take a substantial amount of time to complete. As a result, we may conduct lengthy and expensive clinical trials of our product candidates, only to learn that the product candidate is not an effective treatment or is not superior to existing approved therapies, or has an unacceptable safety profile, which could prevent or significantly delay regulatory approval for such product candidate.

We currently rely on certain third-party manufacturers and other third parties for production of our product candidates and our dependence on these manufacturers may impair the advancement of our research and development programs and the development of our product candidates.

We do not currently have the internal ability to manufacture our product candidates in the quantities that we need to conduct our clinical trials and we rely upon a limited number of manufacturers to supply our product candidates and the components of our drug substances. We also need to rely on manufacturers for the production of our product candidates to support our research and development programs. In addition, we rely on other third parties to perform additional steps in the manufacturing process, including filling and labeling of vials and storage of our product candidates. For the foreseeable future, we expect to continue to rely on contract manufacturers and other third parties to produce product candidates and their components, fill vials, and store sufficient quantities of our product candidates for research and development programs, clinical trials and potential commercial supply. For each of our eteplirsen, Marburg and other development programs, based on limited capacity for our specialized manufacturing needs we have had to enter into limited or, at times, non-exclusive sole-source agreements with multinational manufacturing firms for the production of the active pharmaceutical ingredients (APIs) for eteplirsen, Marburg and other therapeutics. There are a limited number of companies that can produce APIs in the quantities and with the quality and purity that we require. Establishing a relationship with alternative suppliers can be a lengthy process and might cause delays in our development efforts. If we are required to seek alternative supply arrangements, the resulting delays and potential inability to find a suitable replacement could materially and adversely impact our business.

Our product candidates require precise, high-quality manufacturing. 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. Contract drug manufacturers often encounter difficulties involving production yields, quality control and quality assurance and shortages of qualified personnel. If our contract manufacturers or other third parties fail to deliver our product candidates for our research and development programs, clinical use or potential commercial supply on a timely basis, with sufficient quality, and at commercially reasonable prices, and we fail to find replacement manufacturers or to develop our own manufacturing capabilities, we may be required to delay or suspend clinical trials, research and development programs, commercial supply or

otherwise discontinue development and production of our product candidates. In addition, we currently depend on certain third-party vendors, which in some cases may be sole sources, for the supply of raw materials used to produce our product candidates. If the third-party suppliers were to cease production or otherwise fail to supply us with sufficient quantities of quality raw materials and we are unable to contract on acceptable terms for these raw materials with alternative suppliers, if any, our ability to have our product candidates manufactured in sufficient quantities for preclinical testing, clinical trials, and potential commercial use would be adversely affected.

We do not yet have all of the agreements necessary for the supply of APIs and raw materials for the production of any of our product candidates in quantities sufficient for the potential commercial demand and we may not be able to establish or maintain sufficient commercial manufacturing arrangements on commercially reasonable terms. Securing commercial quantities of our product candidates and their components from contract manufacturers will require us to commit significant capital and resources. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties. In addition, contract manufacturers have a limited number of facilities in which our product candidates can be produced and any interruption of the development or operation of those facilities due to events such as order delays for equipment or materials, equipment malfunction or failure or damage to the 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.

Our contract manufacturers are required to produce our clinical product candidates under current good manufacturing practice (cGMP) conditions in order to meet acceptable standards for our clinical trials. If such standards change, the ability of contract manufacturers to produce our product candidates on the schedule we require for our clinical trials may be affected. In addition, contract manufacturers may not perform their agreements with us or may discontinue their business before the time required by us to successfully produce and market our product candidates. We and our contract manufacturers are subject to periodic unannounced inspection 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. 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 approval by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, or cause our products to be recalled or withdrawn.

We may not be able to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing resulting approved drug products, if any.

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for later stage clinical trials in eteplirsen and potential commercialization, we are working to increase the scale of production of our drug product and planning for mid-scale production by the end of 2013. During 2013, we will also evaluate whether to increase API production capacity to a commercial scale which will depend in significant part on feedback from the FDA and our expectations regarding if and when we would commence a pivotal trial for eteplirsen and any subsequent commercialization. In order to conduct larger or late-stage scale clinical trials for a product candidate and supply sufficient commercial quantities of the resulting drug product and its components, if that product candidate is approved for sale, we will need to manufacture it in larger quantities. We may not be able to successfully increase the manufacturing capacity for any of our product candidates, whether in collaboration with third-party manufacturers or on our own, in a timely or cost-effective manner or at all. If a contract manufacturer makes improvements in the manufacturing process for our product candidates, we may not own, or may have to share, the intellectual property rights to those improvements. Significant scale-up of manufacturing may require additional processes, technologies and validation studies, which are costly, may not be successful and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a product candidate itself or of a product candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

In addition, in order to release product and demonstrate stability of product candidates for use in late stage clinical trials (and any resulting drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate our analytical methods or demonstrate adequate stability 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 stability, 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.

We rely on U.S. government contracts to support certain research and development programs and for substantially all of our revenue. If the U.S. government fails to fund such programs on a timely basis or at all, or such contracts are terminated, the results of our operations could be materially and adversely affected.

We rely on U.S. government contracts and awards to fund and support certain development programs, including the Marburg program which accounts for substantially all of our current revenue. The funding of U.S. government programs is subject to Congressional appropriations. Congress generally appropriates funds on a fiscal year basis even though a program may extend over several fiscal years, as is the case with our DoD contract for the development of our Marburg product candidate. Consequently, programs are often only partially funded initially and additional funds are committed only as Congress makes further appropriations. If appropriations for one of our programs become unavailable, or are reduced or delayed, our contracts may be terminated or adjusted by the government, which could have a negative impact on our future revenue under such contract or subcontract. From time to time, when a formal appropriation bill has not been signed into law before the end of the U.S. government's fiscal year, Congress may pass a continuing resolution that authorizes agencies of the U.S. government to continue to operate, generally at the same funding levels from the prior year, but does not authorize new spending initiatives, during a certain period. During such a period, or until the regular appropriation bills are passed, delays can occur in government procurement due to lack of funding and such delays can affect our operations during the period of delay. Currently the DoD is operating under such a continuing resolution for U.S. government fiscal year 2013. Additionally, on March 1, 2013, a sequestration went into effect which implements across-the-board cuts to government agencies, totaling $1.2 trillion over 10 years. These cuts are to be split 50-50 between domestic and defense discretionary spending. The DoD had to make $47 billion in cuts before September 30, 2013. These and other potential budget cuts by the government as well as the effects of government shutdowns could have widespread ramifications including on the DoD's procurement and research and development programs. Sequestration may result in a reduction of funds available for new procurements, but also existing contracts may also be reduced in scope, terminated, or partially terminated. The 2004 Project BioShield Act which created the Special Reserve Fund for use by the Department of Health and Human Services (DHHS) to purchase countermeasures over 10 years mitigates the uncertainty of the annual appropriations process and sequestration, but the $5.6 billion advanced appropriation is rapidly depleting and will expire at the end of U.S. government fiscal year 2013. Thus, the viability of the DHHS and its agencies as a continuing partner and potential customer hinges in part on Congress taking action to replenish the Special Reserve Fund.

In addition, U.S. government contracts generally also permit the government to terminate or renegotiate the contract, in whole or in part, without prior notice, at the government's convenience or for default based on performance. From time to time, we receive communications from the U.S. government regarding our performance, including requests for us to provide additional information and/or take certain steps to remedy noted deficiencies. While we work closely with our contacts at the U.S. government and believe we can adequately address issues raised through such communications, there is no guarantee that we will be able to adequately respond to all requests or remedy all deficiencies cited. If one of our contracts is terminated for convenience, we would generally be entitled to payments for our allowable costs and would receive some allowance for profit on the work performed. If one of our contracts is terminated for default, we would generally be entitled to payments for our work that has been completed to that point. A termination arising out of our default could expose us to liability and have a negative impact on our ability to obtain future contracts. Furthermore, if we fail to satisfy certain performance or deliverable requirements or to adhere to development timelines, revenues associated with the satisfaction of such requirements or timelines may be delayed or may not be realized.

The termination of one or more of these government contracts, whether due to lack of funding, for convenience, for our failure to perform, or otherwise, or the occurrence of delays or product failures in connection with one or more of these contracts, could negatively impact our financial condition. For example, on October 2, 2012, we received notice from the DoD that the program for the development of our Ebola product candidate was terminated for the convenience of the government due to funding constraints. We had previously received a stop-work order for the Ebola program which was in effect from August 2, 2012 through the termination on October 2, 2012. If the government terminates or reduces the Marburg development program or contract, our business could be materially and adversely affected. Furthermore, we can give no assurance that we would be able to procure new U.S. government contracts to offset the revenue lost as a result of termination of any of our existing contracts. Even if our Marburg contract is not terminated and is completed, there is no assurance that we will receive future government contracts.

Even if we successfully complete development of our Marburg and influenza product candidates, the major, if not only, potential purchaser is the U.S. government. The lack of a commercial market makes us reliant upon the U.S. government to determine and communicate the market for biodefense countermeasures and government purchasing is subject to evolving threat assessments and shifting political priorities, which exacerbate market uncertainties. Within the DoD, the war fighter has evolving requirements specifically related to route of administration and time to treat. Until future studies are completed, it is unclear whether our product candidates will successfully meet these requirements. If they do not, the DoD may choose to terminate the contract. With respect to the civilian sector, Marburg and influenza viruses are among the top chemical, biological, radiological and nuclear threats to national security, yet the DHHS has not defined the civilian requirements, making the broader demand for our product candidates uncertain.

This expected dependence on government purchases presents additional challenges, since the government is incentivized to negotiate prices for countermeasures to just above their marginal cost of production, which would severely limit our profit potential. If companies resist low prices, governments can, in extreme cases, threaten compulsory licensing or purchase patent-breaching generics.

Our U.S. government contracts may be terminated and we may be liable for penalties under a variety of procurement rules and regulations and changes in government regulations or practices could adversely affect our profitability, cash balances or growth prospects.

We must comply with laws and regulations relating to the formation, administration and performance of U.S. government contracts, which affect how we do business with our customers. Such laws and regulations may potentially impose added costs on our business and our failure to comply with them may lead to penalties and the termination of our U.S. government contracts. Some significant regulations that affect us include:

- the Federal Acquisition Regulation and supplements, which regulate the formation, administration and performance of U.S. government contracts;
- the Truth in Negotiations Act, which requires certification and disclosure of cost and pricing data in connection with contract negotiations; and
- the Cost Accounting Standards, which impose accounting requirements that govern our right to reimbursement under certain cost-based government contracts.

Our contracts with the DoD are subject to periodic review and investigation. If such a review or investigation identifies improper or illegal activities, we may be subject to civil or criminal penalties or administrative sanctions, including the termination of contracts, forfeiture of profits, the triggering of price reduction clauses, suspension of payments, fines and suspension or debarment from doing business with U.S. government agencies. We could also suffer harm to our reputation if allegations of impropriety were made against us, which would impair our ability to win awards of contracts in the future or receive renewals of existing contracts.

In addition, U.S. government agencies routinely audit and review their contractors' performance on contracts, cost structure, pricing practices and compliance with applicable laws, regulations and standards. They also review the adequacy of, and a contractor's compliance with, its internal control systems and policies, including the contractor's purchasing, property, estimating, compensation and management information systems. Such audits may result in adjustments to our contract costs, and any costs found to be improperly allocated will not be reimbursed. We have recorded contract revenues for the periods presented in this report based upon costs we expect to realize upon final audit; however, we do not know the outcome of any future audits and adjustments and, if future audit adjustments exceed our estimates, our results of operations could be adversely affected. Additionally, we may be required to enter into agreements and subcontracts with third parties, including suppliers, consultants and other third party contractors in order to satisfy our contractual obligations pursuant to our agreements with the DoD. Any such agreement also has to be compliant with the terms of our government grants. Negotiating and entering into such arrangements can be time-consuming and we may not be able to reach agreement with such third parties. Any delay or inability to enter into such arrangements or entering into such arrangements in a manner that is non-compliant with the terms of our grants, may result in violations of our contracts with the DoD.

Clinical trials for our product candidates are expensive and time consuming, may take longer than we expect or may not be completed at all, and their outcomes are uncertain.

We have completed a Phase Ib/II clinical trial for eteplirsen in the UK and announced results in October 2010, which were published in The Lancet in July 2011. We have also completed a U.S.-based Phase IIb placebo controlled trial in eteplirsen and announced results in April 2012. Following completion of this study, we initiated an open label extension study with the same participants from the original Phase IIb placebo controlled trial and announced 48-week results on October 3, 2012, 62-week results on December 7, 2012, 74-week results on April 5, 2013, 84-week results on June 19, 2013 and 96-week results on September 26, 2013. We expect to commence additional trials of eteplirsen and other product candidates in the future based on feedback from the FDA . Each of our clinical trials requires the investment of substantial planning, expense and time, and the timing of the commencement, continuation and completion of these clinical trials may be subject to significant delays relating to various causes including new positions, issues and requests made by the FDA based on scientific developments and data from other drugs being developed by other companies for the treatment of diseases similar to or related to those targeted by our product candidates. Participant enrollment is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, the eligibility criteria for the trial, the existence of competing clinical trials and the availability of alternative or new treatments.

We depend on medical institutions and clinical research organizations (CROs), to conduct our clinical trials in compliance with Good Clinical Practice (GCP) and to the extent they fail to enroll participants for our clinical trials, fail to conduct the study to GCP standards or are delayed for a significant time in the execution of our trials, including achieving full enrollment, we may be affected by increased costs, program delays or both, which may harm our business. In addition, we have in the past conducted clinical trials in foreign countries and may do so again in the future, which may subject us to further delays and expenses as a result of increased drug

shipment costs, additional regulatory requirements and the engagement of foreign CROs, as well as expose us to risks associated with less experienced clinical investigators who are unknown to the FDA, and different standards of medical care. Foreign currency transactions insofar as changes in the relative value of the U.S. dollar to the foreign currency where the trial is being conducted may impact our actual costs. In addition, for some programs, such as DMD and Marburg infection, there are currently no approved drugs to compare against and an agreement about how to measure efficacy has yet to be reached with the FDA and then demonstrated.

Clinical trials must be conducted in accordance with FDA or other applicable foreign government guidelines and are subject to oversight by the FDA, other foreign governmental agencies and IRBs at the medical institutions where the clinical trials are conducted. The FDA or other foreign governmental agencies or we ourselves could delay, suspend or halt our clinical trials of a product candidate for numerous reasons, including:

- Scientific developments and data available for investigational drugs being developed by third parties for the treatment of the same, similar or related diseases to those targeted by our product candidates;
- deficiencies in the trial design;
- deficiencies in the conduct of the clinical trial including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- deficiencies in the clinical trial operations or trial sites resulting in the imposition of a clinical hold;
- the product candidate may have unforeseen adverse side effects, including fatalities, or a determination may be made that a clinical trial presents unacceptable health risks;
- the methods and time required to determine whether the product candidate is effective may take longer than expected;
- fatalities or other adverse events arising during a clinical trial that may not be related to clinical trial treatments;
- the product candidate may appear to be no more effective than current therapies;
- the quality or stability of the product candidate may fail to conform to acceptable standards;
- our inability to produce or obtain sufficient quantities of the product candidate to complete the trials;
- our inability to reach agreement on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- our inability to obtain IRB approval to conduct a clinical trial at a prospective site;
- our inability to obtain regulatory approval to conduct a clinical trial;
- lack of adequate funding to continue the clinical trial, including the occurrence of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with the services of our CROs and other third parties;
- our inability to recruit and enroll individuals to participate in clinical trials for reasons including competition from other clinical trial programs for the same or similar indications; or
- our inability to retain participants who have initiated a clinical trial but may be prone to withdraw due to side effects from the therapy, lack of efficacy or personal issues, or who are lost to further follow-up.

In addition, we may experience significant setbacks in advanced clinical trials, even after promising results in earlier trials, such as unexpected adverse events that occur when our product candidates are combined with other therapies and drugs or given to larger populations, which often occur in later-stage clinical trials. In addition, clinical results are frequently susceptible to varying interpretations by regulatory authorities that may delay, limit or prevent regulatory approvals. Also, patient advocacy groups and parents of trial participants may demand additional clinical trials or continued access to therapies even if our interpretation of clinical results received thus far leads us to determine that additional clinical trials or continued access are unwarranted. Any disagreement with patient advocacy groups or parents of trial participants may require management’s time and attention and may result in legal proceedings being instituted against us, which could be expensive, time-consuming and distracting, and may result in a delay of the program. Negative interpretation of our data by us or regulatory authorities or inconclusive results or adverse medical events, including participant fatalities that may be attributable to our product candidates during a clinical trial may necessitate that it be redesigned, repeated or terminated. Further, some of our clinical trials may be overseen by an independent data and safety monitoring board (DSMB) and a DSMB may determine to delay or suspend one or more of these trials due to safety or futility findings based on events occurring during a clinical trial. Any such delay, suspension, termination or request to repeat or redesign a trial could increase our costs and prevent or significantly delay our ability to commercialize our product candidates.

The Animal Rule is a seldom-used approach to seeking approval of a new drug and our infectious disease program may not meet the requirements for this path to regulatory approval.

Clinical trials cannot be used to assess the efficacy of most biodefense countermeasures against rare and lethal pathogens due to ethical considerations and the relative infrequency of naturally occurring cases. In the United States, we plan to develop the therapeutic product candidate to treat Marburg virus using the Animal Rule regulatory mechanism. Pursuant to the Animal Rule, the sponsor of a drug product must demonstrate efficacy in animal models and safety in humans. There is no guarantee that the FDA will agree to this approach to the development of our infectious disease product candidate, considering that no validated animal model has been established as predicting human outcomes in the prevention or treatment of any filovirus disease. Animal models represent, at best, a rough approximation of efficacy in humans, and, as such, countermeasures developed using animal models will be untested until their use in humans during an emergency. We have yet to demonstrate the predictive value of our animal studies to the FDA's satisfaction. If we fail to do so, we will have to demonstrate efficacy of AVI-7288 through adequate well-controlled trials in humans in order to obtain regulatory approval of this product in the United States, which, if possible, will greatly add to the time and expense required to commercialize this product. Furthermore, the Animal Rule mechanism has been used only rarely and questions remain regarding the FDA's interpretation and implementation. Only one novel product has been approved using the Animal Rule. It has thus far been used to extend the indicated use of three previously approved products which had considerable prior human experience. We do not have any experience successfully navigating this approach to drug approval. Even if the Animal Rule represents a viable approach to seeking approval of AVI-7288, it may present challenges for gaining final regulatory approval for this product candidate, including an extended timeline to approval and less predictable study requirements. In addition, the FDA would require post-marketing human efficacy studies if the countermeasure is used in humans, which would most likely be in the aftermath of a bioterrorist attack. The ability to reliably perform efficacy clinical trials in the midst of a national crisis is uncertain.

The timing and conduct of animal studies may be further constrained given that filoviruses are classified for use only in BSL-4 laboratories. There are limited laboratories and staff world-wide that can work with these live viruses and companies will be competing for the limited availability of this critical infrastructure to test their countermeasures. Furthermore, we anticipate limits in conforming to good laboratory practice (GLP) requirements given the requirement for BSL-4 containment.

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

We had an operating loss of $57.4 million for the nine months ended September 30, 2013 and incurred an operating loss of $29.7 million for the year ended December 31, 2012. As of September 30, 2013, our accumulated deficit was $534.4 million and substantially all of our revenues to date have been derived from research and development contracts with the DoD. 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;
- 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 likely need additional funds to conduct our planned research and development 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, it 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, through September 30, 2013, we sold an aggregate of approximately 3.4 million shares of our common stock in connection with our July 2013 ATM equity offering program. 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 or to conduct clinical trials and to market our product candidates. Other than pre-clinical 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 other than that with the U.S. government. 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.

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 and 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.

Our RNA-based, or 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 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. We are conducting toxicology, pharmacology, pharmacokinetics and other preclinical studies and, although we have conducted Phase I clinical trials for AVI-6003 (we are now pursuing development of AVI-7288, one of the two component oligomers in AVI-6003) and AVI-7100 and conducted a Phase IIb clinical trial in eteplirsen, additional preclinical studies may be required for these product candidates and before other product candidates enter human clinical trials. In addition, preclinical models to study participant toxicity and activity of compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease and there may be substantially different results in clinical trials from the results obtained in preclinical studies. Any failures or setbacks in utilizing our PMO-based technology, including adverse effects resulting from the use of this technology 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.

If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth, ability to perform our U.S. government contracts 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 and personnel with experience overseeing compliance with and execution of the terms of our U.S. government contracts. The loss of the services of any one of the principal members of our managerial, scientific or government contract compliance staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field and for qualified personnel with government contracting experience 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 managerial, scientific and government contract compliance staff. In certain instances, we may also need to expand our workforce and our management ranks. We face intense competition for qualified individuals from numerous pharmaceutical and biotechnology companies, as well as academic and other research institutions. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our proprietary programs and perform our U.S. government contracts would be adversely affected. Any failure to perform under our U.S. government contracts could result in a termination of the agreement, which would harm our business.

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 revenues, 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:

- our patent rights might be challenged, invalidated, or circumvented, or otherwise might not provide any competitive advantage;
- as a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection for disease treatments that prove successful; and
- jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., 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 U.S. Supreme Court has issued decisions, the full impact of which 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 (cDNA) 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, 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 Holding B.V. (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, 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 our drug eteplirsen in the European Union would infringe on such patent. Both we and Prosensa are appealing the Opposition Division decision given that the decision could have a substantial effect on our businesses 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 certain patent claims that Prosensa has rights to, and others that it is pursuing, in other jurisdictions, including Japan and the United States, that may provide the basis for Prosensa or other parties that have rights to these claims to assert that commercialization of our drug eteplirsen in such other jurisdictions would infringe on such claims.

The DMD patent landscape is continually evolving and multiple parties, 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 those 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 program.

We face intense competition and rapid technological change, which may result in others discovering, developing or commercializing competing products before or more successfully than we do.

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, Isis Pharmaceuticals and Santaris Pharma A/S (Santaris) share a focus on RNA-based drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include Prosensa and GlaxoSmtihKline (GSK) and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs.

Although Prosensa/ GSK recently announced that the primary endpoint for their lead DMD drug candidate was not met, we may still face competitive risks arising from the Prosensa/ GSK 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 clinical trial 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, and the sale of any approved products in the future, may expose us to liability claims. 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 biohazardous 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 conditions. We expect that our operations will be affected by other new environmental and 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 cybersecurity 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 liability and our research and development programs and the development of our product candidates could be delayed.

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. For example, during 2012, our stock traded from a low of $3.30 per share to a high of $45.00 per share. As an additional example, we note that on July 24, 2013 our stock price decreased 19% on the same day that we made an announcement regarding eteplirsen and recent communications we had with the FDA. 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:

- The timing of our filings with the regulatory authorities and regulatory decisions and developments including the probability of a decision by the FDA to review eteplirsen on an accelerated or normal pathway, if at all.
- positive or negative results 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 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 government regulations or requirements by regulatory in the approval process;
- developments concerning proprietary rights, including patents and litigation matters;
- public concern relating to the commercial value 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;
- litigation; 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, if instituted against us, 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 quarterly 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. Some of these fluctuations may be very pronounced such as in the case of the impact to our operating results as a result of our warrant offerings in January and August 2009 of which warrants for an aggregate of 1.0 million shares remain outstanding and exercisable as of September 30, 2013. Each of these warrants is classified as a derivative liability and accordingly, the fair value of the warrants is recorded on our condensed consolidated balance sheet as a liability, and such fair value is adjusted at each financial reporting date with the adjustment to fair value reflected in our condensed consolidated statement of operations and comprehensive income (loss). For example, for the nine months ended September 30, 2013, the impact of the change in fair value of these warrants resulted in a $46.0 million charge in our unaudited condensed consolidated statement of operations and comprehensive income (loss). The fair value of the warrants is determined using the Black-Scholes-Merton option valuation model. Fluctuations in the assumptions and factors used in the Black-Scholes-Merton model can result in adjustments to the fair value of the warrants reflected on our balance sheets and, therefore, our statement of operations. Due to the classification of such warrants and other factors, quarterly results of operations are difficult to forecast, and period-to-period comparisons of our operating results may not be predictive of future performance. Additionally, our quarterly operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Specifically, a change in the timing of activities performed in support of our U.S. government research contracts could either accelerate or defer anticipated revenue from period to period. 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 quarters, 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, 2013, there were 37.5 million shares of common stock outstanding, outstanding awards to purchase 4.3 million shares of common stock under various incentive stock plans and outstanding warrants to purchase up to 1.0 million shares of common stock. Additionally, as of September 30, 2013, there were 3.0 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan and 250,000 shares of common stock remain available for issuance under the Company’s 2013 Employee Stock Purchase Plan. In addition, 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. 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.

On November 7, 2013, the Company separately entered into the Company's standard Senior Vice President Change in Control and Severance Agreement (the "***Change in Control Agreement***") with Edward Kaye, the Company's Senior Vice President, Chief Medical Officer.

The Change in Control Agreement provides that if the executive experiences certain terminations during the 12-month period following a change in control, and if the executive delivers to the Company a general release of claims that becomes effective and irrevocable within 60 days following such covered termination, then in addition to any accrued but unpaid salary, bonus, vacation and expense reimbursement payable in accordance with applicable law, the Company shall provide such executive with the following: (i) cash payment equal to 18 months of such executive's base salary at the rate in effect immediately prior to such executive's termination of employment payable in a cash lump sum, less applicable withholdings; (ii) cash payment equal to 100% of his annual target bonus assuming achievement of performance goals at 100% payable in a cash lump sum, less applicable withholdings; (iii) accelerated vesting on 100% of his outstanding and unvested equity awards; and (iv) if such executive elects to receive continued healthcare coverage pursuant to the provisions of COBRA, the Company shall directly pay, or reimburse such executive for, the premium for such executive and his covered dependents through the earlier of (a) the 18-month anniversary of the date of his termination of employment and (b) the date he and his covered dependents, if any, become eligible for healthcare coverage under another employer's plan(s).

The foregoing description of the terms of the Change in Control Agreement does not purport to be a complete description and is qualified in its entirety by reference to the form of Senior Vice President Change in Control and Severance Agreement that is filed as Exhibit 10.19 to the Company's Annual Report on Form 10-K for the year ended December 31, 2012.

Item 6. Exhibits.

Exhibit Number	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

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 12, 2013

By: /s/ CHRISTOPHER GARABEDIAN
Christopher Garabedian
President and Chief Executive Officer

Date: November 12, 2013

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Senior Vice President, Chief Financial Officer (Principal Financial and Accounting Officer)

EXHIBIT INDEX

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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

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 12, 2013

/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 12, 2013

/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, 2013, 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 12, 2013

/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, 2013, 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 12, 2013

/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.