

Sarepta Therapeutics, Inc.
Form 10-Q
November 07, 2012
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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, 2012

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)

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Oregon
(State or other jurisdiction of
incorporation or organization)

93-0797222
(I.R.S. Employer
Identification No.)

3450 Monte Villa Parkway, Suite 101, Bothell, Washington
(Address of principal executive offices)

98021
(Zip Code)

Registrant's telephone number, including area code: (425) 354-5038

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
(Class)

25,452,785
(Outstanding as of October 31, 2012)

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SAREPTA THERAPEUTICS, INC.

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Table of Contents**PART I FINANCIAL INFORMATION****Item 1. Financial Statements.**

SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

BALANCE SHEETS

(unaudited)

(in thousands, except per share data)

	September 30, 2012	December 31, 2011
Assets		
Current Assets:		
Cash and cash equivalents	\$ 37,987	\$ 39,904
Accounts receivable	2,474	3,633
Other current assets	1,698	1,647
Total Current Assets	42,159	45,184
Property and Equipment, net of accumulated depreciation and amortization of \$16,444 and \$15,765	3,661	4,265
Patent Costs, net of accumulated amortization of \$2,521 and \$2,199	4,837	4,764
Other assets	2,425	155
Total Assets	\$ 53,082	\$ 54,368
Liabilities and Shareholders Equity		
Current Liabilities:		
Accounts payable	\$ 4,334	\$ 9,396
Accrued employee compensation	2,180	2,244
Long-term debt, current portion	88	85
Warrant valuation	45,209	5,446
Deferred revenue	3,304	3,304
Other liabilities	85	126
Total Current Liabilities	55,200	20,601
Commitments and Contingencies		
Long-term debt, non-current portion	1,690	1,757
Other long-term liabilities	758	993
Shareholders Equity:		
Preferred stock, \$.0001 par value, 3,333,333 shares authorized; none issued and outstanding	0	0
Common stock, \$.0001 par value, 50,000,000 shares authorized; 24,302,261 and 22,623,853 issued and outstanding	2	2
Additional paid-in capital	364,616	340,979
Deficit accumulated during the development stage	(369,184)	(309,964)
Total Shareholders Equity	(4,566)	31,017

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Total Liabilities and Shareholders	Equity	\$	53,082	\$	54,368
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See accompanying notes to financial statements.

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SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

STATEMENTS OF OPERATIONS and COMPREHENSIVE INCOME (LOSS)

(unaudited)

(in thousands, except per share data)

July 22, 1980

	Three months ended September 30,		Nine months ended September 30,		(Inception) through
	2012	2011	2012	2011	September 30, 2012
Revenues from license fees, grants and research contracts	\$ 7,574	\$ 7,524	\$ 29,993	\$ 33,405	\$ 166,212
Operating expenses:					
Research and development	10,914	15,610	39,568	48,161	372,834
General and administrative	3,565	3,185	9,761	12,171	114,218
Acquired in-process research and development	0	0	0	0	29,461
Operating loss	(6,905)	(11,271)	(19,336)	(26,927)	(350,301)
Other income (loss):					
Interest income and other, net	67	199	270	440	9,439
Gain (loss) on change in warrant valuation	(42,716)	7,052	(40,154)	25,579	(15,184)
Realized gain on sale of short-term securities available-for-sale	0	0	0	0	3,863
Write-down of short-term securities available-for-sale	0	0	0	0	(17,001)
	(42,649)	7,251	(39,884)	26,019	(18,883)
Net income (loss)	\$ (49,554)	\$ (4,020)	\$ (59,220)	\$ (908)	\$ (369,184)
Other comprehensive income (loss):					
Write-down of short-term securities available-for-sale	0	0	0	0	17,001
Realized gain on sale of short-term securities available-for-sale	0	0	0	0	(3,863)
Unrealized loss on short-term securities available-for-sale	0	0	0	0	(13,138)
	0	0	0	0	0
Comprehensive income (loss)	\$ (49,554)	\$ (4,020)	\$ (59,220)	\$ (908)	\$ (369,184)
Net income (loss) per share - basic	\$ (2.17)	\$ (0.18)	\$ (2.61)	\$ (0.04)	
Net income (loss) per share - diluted	\$ (2.17)	\$ (0.18)	\$ (2.61)	\$ (0.04)	
Weighted average number of common shares outstanding for computing basic income (loss) per share (in thousands)	22,824	22,623	22,691	21,254	
	22,824	22,623	22,691	21,254	

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Weighted average number of common shares outstanding
for computing diluted income (loss) per share (in
thousands)

See accompanying notes to financial statements.

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SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	For the Period		
	Nine months ended September 30,	September 30,	July 22, 1980 (Inception) through September 30, 2012
	2012	2011	
Cash flows from operating activities:			
Net income (loss)	\$ (59,220)	\$ (908)	\$ (369,184)
Adjustments to reconcile net income (loss) to net cash flows used in operating activities:			
Depreciation and amortization	1,090	763	21,535
Loss on disposal of assets	182	161	2,453
Realized gain on sale of short-term securities available-for-sale	0	0	(3,863)
Write-down of short-term securities available-for-sale	0	0	17,001
Impairment charge on real estate owned	0	109	1,445
Stock-based compensation	1,840	2,454	30,835
Conversion of interest accrued to common stock	0	0	8
Acquired in-process research and development	0	0	29,461
Increase (decrease) on warrant liability	40,154	(25,579)	15,184
(Increase) in accounts receivable, other current assets and other assets	(1,162)	(1,356)	(6,336)
Increase (decrease) in accounts payable, accrued employee compensation, and other liabilities	(5,420)	6,325	8,711
Net cash used in operating activities	(22,536)	(18,031)	(252,750)
Cash flows from investing activities:			
Purchase of property and equipment	(108)	(973)	(19,987)
Patent costs	(614)	(548)	(10,106)
Purchase of marketable securities	0	0	(112,993)
Sale of marketable securities	0	0	117,724
Acquisition costs	0	0	(2,389)
Net cash used in investing activities	(722)	(1,521)	(27,751)
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	21,405	32,380	319,283
Repayments of long-term debt	(64)	(61)	(409)
Buyback of common stock pursuant to rescission offering	0	0	(289)
Withdrawal of partnership net assets	0	0	(177)
Issuance of convertible debt	0	0	80
Net cash provided by (used in) financing activities	21,341	32,319	318,488
Increase (decrease) in cash and cash equivalents	(1,917)	12,767	37,987
Cash and cash equivalents:			

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Beginning of period	39,904	33,589	0
End of period	\$ 37,987	\$ 46,356	\$ 37,987

SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:

Cash paid during the year for interest	\$ 65	\$ 68	\$ 554
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SUPPLEMENTAL SCHEDULE OF NONCASH INVESTING ACTIVITIES AND FINANCING ACTIVITIES:

Short-term securities available-for-sale received in connection with the private offering	\$ 0	\$ 0	\$ 17,897
Issuance of common stock and warrants in satisfaction of liabilities	\$ 391	\$ 643	\$ 1,579
Issuance of common stock for building purchase	\$ 0	\$ 0	\$ 750
Assumption of long-term debt for building purchase	\$ 0	\$ 0	\$ 2,200
Issuance of common stock to acquire assets	\$ 0	\$ 0	\$ 8,075
Assumption of liabilities to acquire assets	\$ 0	\$ 0	\$ 2,124

See accompanying notes to financial statements.

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SAREPTA THERAPEUTICS, INC.

NOTES TO FINANCIAL STATEMENTS

(Unaudited)

1. ORGANIZATION AND BASIS OF PRESENTATION

Sarepta Therapeutics, Inc., formerly AVI BioPharma, Inc., is a biopharmaceutical company incorporated in the State of Oregon on July 22, 1980. On July 10, 2012, the shareholders approved a proposal to change the name of the company to Sarepta Therapeutics, Inc. (Sarepta or the Company) and the change was effective on July 11, 2012.

The Company is 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 primarily focused on rapidly advancing the development of its Duchenne muscular dystrophy drug candidates, including its lead product candidate, eteplirsen, which is currently in a Phase IIb clinical trial. The Company is also focused on developing therapeutics for the treatment of infectious diseases, including its lead infectious disease programs aimed at the development of drug candidates for the Ebola and Marburg hemorrhagic fever viruses for which the Company has historically received and expects to continue to receive significant financial support from U.S. government research contracts.

The Company effected a one-for-six reverse stock split of its outstanding common stock on July 11, 2012. The accompanying unaudited condensed consolidated financial statements and related notes to the unaudited condensed consolidated financial statements give retroactive effect to the reverse stock split for all periods presented.

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, 2011 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.

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

The accompanying unaudited condensed consolidated financial statements reflect all adjustments that are, in the opinion of management, necessary for a fair presentation of the financial position, results of operations and cash flows for the interim periods. 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, 2011. The results of operations for the interim periods presented are not necessarily indicative of the results to be expected for the full year.

Since its inception in 1980, the Company has incurred losses of approximately \$369.2 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and acquired in-process research and development resulting from two acquisitions. The Company has 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 the Company does achieve revenue from product sales, the Company expects to incur operating losses over the next several years.

In the periods presented, substantially all of the revenue generated by the Company was derived from research contracts with the U.S. government. As of September 30, 2012, the Company had completed all of its contracts with the U.S. government except for the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses and the contract for intramuscular injection (IM contract). On August 2, 2012, the Company received a stop-work order related to the Ebola virus portion of the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses and on October 2, 2012, the U.S. government terminated the Ebola portion of the contract due to funding constraints. The stop-work order and subsequent termination does not apply to the Company's ongoing Marburg activities which includes the IM contract. See Note 6 U.S. Government Contracts for additional information.

As of September 30, 2012, cash and cash equivalents were \$38.0 million. The Company's principal sources of liquidity have been equity financings and revenue from U.S. government research contracts. The Company anticipates receiving continued funding from the U.S.

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government to pursue the development of the Company's therapeutics against the Marburg virus.

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In September 2012, the Company entered into a financing agreement where the Company may issue common stock at the then current market price up to a total \$40.0 million. As of September 30, 2012, the Company had issued approximately 1.4 million shares of stock and received net proceeds of \$19.9 million under the financing arrangement. Subsequent to September 30, 2012 and up to October 31, 2012, the Company sold an additional 0.6 million shares under the agreement and received net proceeds of \$16.4 million. The Company is likely to pursue additional funding through other public or private financings and generate cash by establishing collaborations or licensing its product candidates.

The Company's principal uses of cash are research and development expenses, general and administrative expenses and other working capital requirements. Many of these uses of cash are discretionary in nature and can be significantly reduced at the direction of the Company's management and Board of Directors. The Company believes these sources of cash and potential reductions in discretionary spending, when combined together, would provide sufficient cash to fund the Company's operations for at least the following 12 months. Should the Company's funding from the U.S. government cease or be delayed, it would have a negative impact on the Company's financial condition and the Company would significantly reduce research and development efforts associated with therapeutics for the Marburg virus.

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

As of the date of this report, the Company is not a party to any material legal proceedings with respect to itself, its subsidiaries, or any of its material properties. 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, effects from the use of therapeutics utilizing its technology, professional services or others. It is impossible to predict whether any resulting liability would have a material adverse effect on the Company's financial position, results of operations or cash flows.

Reclassifications

Certain inception to date amounts have been reclassified to conform to current year presentation. These changes did not have a significant impact on the Company's net loss, assets, liabilities, shareholders' equity (deficit) or cash flows.

2. NET INCOME (LOSS) PER SHARE

Basic net income (loss) per share is computed by dividing net income (loss) by the weighted-average number of common shares outstanding. Diluted net income (loss) per share is computed by dividing net income (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,	
	2012	2011	2012	2011
	(in thousands, except per share data)		(in thousands, except per share data)	
Net income (loss)	\$ (49,554)	\$ (4,020)	\$ (59,220)	\$ (908)
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	22,824	22,623	22,691	21,254
Dilutive effect of warrants and stock options after application of the treasury stock method*				
Weighted-average number of common shares outstanding for computing diluted earnings per share	22,824	22,623	22,691	21,254

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Net Income (loss) per share	basic	\$	(2.17)	\$	(0.18)	\$	(2.61)	\$	(0.04)
Net Income (loss) per share	dilutive	\$	(2.17)	\$	(0.18)	\$	(2.61)	\$	(0.04)

* Warrants, stock options, restricted stock units and stock appreciation rights to acquire 7,371,471 and 7,463,668 shares of common stock were excluded from the net income (loss) per share calculation for the three months and nine months ended September 30, 2012 and 2011, respectively, as their effect would have been anti-dilutive.

Table of Contents**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, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Cash and Cash equivalents	\$ 37,987	\$ 37,987	\$	\$
Total assets	\$ 37,987	\$ 37,987	\$	\$

	Fair Value Measurement as of September 30, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	\$ 45,209	\$	\$	\$ 45,209
Total liabilities	\$ 45,209	\$	\$	\$ 45,209

	Fair Value Measurement as of December 31, 2011			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Cash and Cash equivalents	\$ 39,904	\$ 39,904	\$	\$
Total assets	\$ 39,904	\$ 39,904	\$	\$

	Fair Value Measurement as of December 31, 2011			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	\$ 5,446	\$	\$	\$ 5,446
Total liabilities	\$ 5,446	\$	\$	\$ 5,446

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

The carrying amounts reported in the balance sheets for accounts receivable, accounts payable, and other current and noncurrent monetary assets and liabilities 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 because of similar characteristics to other debt instruments with comparable risk.

Table of Contents**4. ACCOUNTS RECEIVABLE**

Accounts receivable are stated at invoiced amount and do not bear interest. Because all accounts receivable are from the U.S. government and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. The accounts receivable balance included \$2,474,000 and \$2,093,000 of U.S. government receivables that were unbilled at September 30, 2012 and December 31, 2011, respectively.

5. WARRANTS

Warrants issued in connection with the Company's December 2007, January 2009, and August 2009 common stock offerings are classified as liabilities as opposed to equity due to their settlement terms which require settlement in registered shares. 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 fair value of the warrants classified as liabilities was recorded on the balance sheet at issuance and is adjusted to fair value at each financial reporting period, with changes in the fair value recorded as a gain or loss in the statement of operations. The fair value is determined using the Black-Scholes 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:

	Three and Nine Months Ended September 30,	
	2012	2011
Risk-free interest rate	0.1%-0.3%	0.1%-1.3%
Expected dividend yield	0%	0%
Expected lives	0.2-1.9 years	1.2-3.4 years
Expected volatility	100.7%-209.1%	55.3%-88.5%
Shares underlying warrants classified as liabilities	4,784,519	4,824,827
Market value of stock at beginning of year	\$ 4.50	\$ 12.72
Market value of stock at end of period	\$ 15.53	\$ 6.72

A reconciliation of the change in value of the Company's warrant liability for the three and nine months ended September 30, 2012 is as follows:

	Three Months Ended	Nine Months Ended
	September 30,	September 30,
	2012	2012
	(in thousands)	(in thousands)
Balance at beginning of period	\$ 2,884	\$ 5,446
Increase (Decrease) in value of warrants	42,716	40,154
Reclassification to shareholders' equity upon exercise of warrants	(391)	(391)
Balance at September 30, 2012	\$ 45,209	\$ 45,209

For the three months and 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 information about warrants outstanding at September 30, 2012.

Exercise Price	Outstanding Warrants at September 30, 2012	Expiration Date	Weighted Average Remaining Contractual Life	Exercisable Warrants
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			(Years)	
\$0.0018	2,778	No expiration date	No expiration date	2,778
6.84	167	No expiration date	No expiration date	167
6.96	2,317,281	7/30/2014	2.0	2,317,281
8.70	7,468	1/30/2014	1.0	7,468
10.68	1,568,385	8/31/2014	2.0	1,568,385
14.70	891,385	12/18/2012	0.2	891,385
21.66	34,626	11/13/2012	0.1	34,626
	4,822,090			4,822,090

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Subsequent to September 30, 2012, and through October 31, 2012, 531,913 warrants were exercised at a weighted average exercise price of \$10.56 generating proceeds of \$5.6 million.

6. U.S. GOVERNMENT CONTRACTS

The Company recognizes revenues from U.S. government research contracts during the period in which the related expenditures are incurred and present these revenues and related expenses gross in the consolidated financial statements. In the periods presented, substantially all of the revenue generated by the Company was derived from research contracts with the U.S. government. As of September 30, 2012, the Company had completed all of its contracts with the U.S. government except for the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses and the contract for intramuscular injection (IM contract). On October 2, 2012, the U.S. government terminated the Ebola portion of the July 2010 agreement.

The following table sets forth the revenue for each of the contracts with the U.S. government for the three months and nine months ended September 30, 2012 and 2011.

	Three Months Ended September 30, 2012 2011 (in thousands)		Nine Months Ended September 30, 2012 2011 (in thousands)	
July 2010 Agreement (<i>Ebola and Marburg</i>)	\$ 7,511	\$ 7,290	\$ 29,844	\$ 29,780
August 2012 Agreement (<i>Intramuscular</i>)	50		50	
June 2010 Agreement (<i>H1N1</i>)		183		3,390
Other Agreements	13	51	99	235
Total	\$ 7,574	\$ 7,524	\$ 29,993	\$ 33,405

July 2010 Agreement (*Ebola and Marburg*)

On July 14, 2010, the Company was awarded a contract with the U.S. Department of Defense, or DoD, Chemical and Biological Defense Program through the U.S. Army Space and Missile Defense Command 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. During 2012, the Company received permission from the Food and Drug Administration (FDA) to proceed with single oligomers, AVI-7537 and AVI-7288, as the lead product candidates against the Ebola and Marburg virus infections, respectively, and, in June 2012, requested a contract modification from DoD to proceed with these single oligomers as the lead product candidates against the Ebola and Marburg virus infections.

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 U.S. government terminated the Ebola portion of the contract for the convenience of the government due to recently imposed funding constraints. The Ebola portion of the contract with the DoD represented approximately half of the contract revenue. The Company anticipates a reduction in associated research and development costs as research on the Ebola virus therapeutic candidate will be substantially curtailed without further funding. These research and development costs are primarily with third party contractors. The Company anticipates that the impact to general and administrative expenses will be minimal.

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. After completion of the first segment, and each successive segment, DoD has the option to proceed to the next segment. Activities under the first segment began in July 2010 and include Phase I studies in healthy volunteers as well as preclinical studies which are scheduled to be completed in 2013. The remaining funding as of September 30, 2012 for the current Marburg segment is approximately \$19.5 million. If DoD exercises its options for segments II, III and IV for AVI-7288, contract activities would include all clinical and licensure activities necessary to obtain Food and Drug Administration (FDA) regulatory approval. The funding for segments II, III and IV of the Marburg virus portion of contract is estimated to be approximately \$84.4 million.

Table of Contents**August 2012 Agreement (Intramuscular administration)**

On August 29, 2012, the Company was awarded a new contract from the U.S. Department of Defense's Joint Project Manager Transformational Medical Technologies (JPM-TMT) program, a component of the U.S. Department of Defense's Joint Program Executive Office for Chemical and Biological Defense. The contract provides funding to the Company of 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 evaluation is scheduled to conclude in the second half of 2013. Under the July 2010 Agreement (Ebola and Marburg) described above, the Company is developing AVI-7288 as an intravenous formulation.

June 2010 Agreement (H1N1/Influenza)

On June 4, 2010, the Company entered into a contract with the Defense Threat Reduction Agency to advance the development of AVI-7100 as a medical countermeasure against the pandemic H1N1 influenza virus in cooperation with the Transformational Medical Technologies program of DoD. The period of performance for this contract ended on June 3, 2011.

7. STOCK COMPENSATION**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 on the first anniversary of the grant and the remaining underlying shares vesting pro-ratably on a monthly basis thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant. As of September 30, 2012, 1,539,930 shares of common stock remain available for future grant.

A summary of the Company's stock option activity with respect to the nine months ended September 30, 2012 follows:

Stock Options	Underlying Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term	Aggregate Intrinsic Value
Outstanding at December 31, 2011	2,417,659	\$ 11.18		
Granted	921,790	9.19		
Exercised	(167,341)	7.28		
Canceled or expired	(731,638)	11.32		
Outstanding at September 30, 2012	2,440,470	\$ 10.65	8.01	\$ 14,315,000
Vested at September 30, 2012 and expected to vest	2,325,299	\$ 10.75	7.93	\$ 13,547,000
Exercisable at September 30, 2012	770,611	\$ 14.54	5.20	\$ 3,174,000

The weighted-average fair value per share of stock-based awards granted to employees during the three months ended September 30, 2012 and 2011 was \$7.09 and \$4.74, respectively, and during the nine months ended September 30, 2012 and 2011 was \$6.52 and \$6.54, respectively. During the nine months ended September 30, 2012 and 2011, the total intrinsic value of stock options exercised was \$982,000 and \$82,000 respectively, and the total grant date fair value of stock options that vested was \$3,194,000 and \$2,300,000, respectively.

Valuation Assumptions

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

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

	Three and Nine Months Ended September 30,	
	2012	2011
Risk-free interest rate	0.6%-1.1%	1.1%-2.4%
Expected dividend yield	0%	0%
Expected lives	5.1-5.3 years	5.4-5.5 years
Expected volatility	79.7%-94.8%	78.2%-81.6%

Restricted Stock Units

In April 2012, the Company granted 32,377 shares of restricted stock units (RSUs) to employees in lieu of cash for a portion of the 2012 bonus. These shares vest over a two-year period and have a weighted average grant date fair value of \$5.40 per share. In addition, in August 2012, 7,500 RSUs with a grant date fair value of \$10.08 per share were granted to an officer of the Company. The weighted-average grant-date fair value of RSU awards is based on the market price of the Company's common stock on the date of grant. The following table sets forth restricted stock unit activity for the period shown:

Restricted Stock Units	Nine Months Ended September 30, 2012	
	Shares	Weighted Average Grant Date Fair Value per Share
Outstanding at December 31, 2011		\$
Granted	39,877	6.28
Vested		
Forfeited or canceled	(966)	5.40
Outstanding at September 30, 2012	38,911	\$ 6.30

Stock Appreciation Rights

In August 2012, the Company issued 70,000 stock appreciation rights (SARs) to the Company's president & CEO. The SARs vest over 4 years, have an exercise price of \$10.08 per share and have a grant date fair value of \$508,000. The outstanding SARs are classified as equity because the agreement requires settlement in shares of stock.

Stock-based Compensation Expense

A summary of the stock-based compensation expense, including options, restricted stock units, stock appreciation rights and restricted stock, recognized in the statements of operations is as follows:

	Three Months Ended		Nine Months Ended	
	September 30, 2012	September 30, 2011	September 30, 2012	September 30, 2011
	(in thousands)		(in thousands)	
Research and development	\$ 271	\$ 266	\$ 783	\$ 998
General and administrative	421	326	1,057	1,456
Total	\$ 692	\$ 592	\$ 1,840	\$ 2,454

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As of September 30, 2012, there was \$10,305,000 of unrecognized compensation cost related to non-vested share-based compensation arrangements outstanding including stock options, restricted stock units, stock appreciation rights and restricted stock. These costs are expected to be recognized over a weighted-average period of 3.2 years.

Table of Contents**8. INCOME TAXES**

At December 31, 2011, the Company had net deferred tax assets of approximately \$116.8 million. The net deferred tax assets are primarily composed of U.S. federal and state tax net operating loss 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 net operating loss and research and development credit carryforwards to offset future taxable income based on ownership changes and the value of the Company's stock.

9. RESTRUCTURING

In December 2011, the Company restructured its operations by reducing its workforce by 28%. Restructuring charges totaling \$1,145,000 were recorded in 2011 and included severance and related costs. The restructuring was completed by January 31, 2012 and all severance costs are expected to be paid by December 31, 2012.

Changes in the liability and the balance related to the December 2011 restructuring plan are as follows:

	Nine Months Ending September 30, 2012 (in thousands)
Balance at December 31, 2011	\$ 828
Restructuring charge for severance	
Severance payments	(579)
Balance at September 30, 2012	\$ 249

10. RECENT ACCOUNTING PRONOUNCEMENTS

In April 2011, the Financial Accounting Standards Board (FASB) issued guidance to achieve common fair value measurement and disclosure requirements between GAAP and International Financial Reporting Standards. This guidance amends current fair value measurement and disclosure guidance to include increased transparency around valuation inputs and investment categorization. The guidance is effective for fiscal years and interim periods beginning after December 15, 2011. The adoption of this new guidance did not have a material impact on the Company's financial statements.

In June 2011, the FASB issued guidance regarding presentation of other comprehensive income in the financial statements. This guidance eliminated the option under GAAP to present other comprehensive income in the statement of changes in equity. Under the guidance, the Company had the option to present the components of net income and comprehensive income in either one or two consecutive financial statements. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011. The Company elected to present the components of net income and comprehensive income in one financial statement and the adoption of this new guidance did not have a material impact on the Company's financial statements.

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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, 2011 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 and Section 21E of the Exchange Act. All statements other than historical or current facts, including, without limitation, statements about our business strategy, plans and objectives of management, and our future prospects, are forward-looking statements and are sometimes identified by such words as "believe," "expect," "anticipate," "may," "will," "should," "could," "would," "plan," "estimate," "project," "predict," and "potential," and words of similar import. Forward-looking statements include, but are not limited to, statements regarding:

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 initiation of enrollment of a pivotal Phase III trial in late 2013;

our expectations regarding the timing, completion and receipt of results from our ongoing development programs;

the receipt of any required approval from the U.S. Food and Drug Administration, or FDA, or other regulatory approval for our products;

the effect of regulation by FDA and other agencies;

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; and

our expectations about funding from the government and other sources.

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.

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

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dystrophy 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 program funded by the U.S. government 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 Duchenne muscular dystrophy, or DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no disease-modifying therapies available for DMD. Eteplirsen is our lead therapeutic candidate for DMD and if we are successful in our development efforts, eteplirsen will address a severe unmet medical need. We recently completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study, we initiated an open label extension study with the same participants from the original Phase IIb placebo controlled trial.

In April 2012, we announced the results from our DMD Phase IIb clinical trial which determined that treatment with eteplirsen met the primary efficacy endpoint in the Phase IIb study. Eteplirsen administered once weekly at 30 mg/kg over 24 weeks resulted in a statistically significant ($p \leq 0.002$) increase in novel dystrophin (22.5% dystrophin-positive fibers as a percentage of normal) compared to no increase in the placebo group. Restoration of dystrophin expression and dystrophin positive fibers is believed to be critical for successful disease-modifying treatment of individuals with DMD. In the study, a shorter duration of eteplirsen treatment, 12 weeks, did not show a significant increase in novel dystrophin (0.79% dystrophin-positive fibers as a percentage of normal; p-value NS), despite administration of the drug at a higher dose (50 mg/kg once weekly). No significant improvements in clinical outcomes in the treated groups were observed compared to placebo.

On July 24, 2012, we announced interim results from our DMD open label extension study which indicated that treatment with eteplirsen over 36 weeks achieved a significant clinical benefit on the primary clinical outcome, the 6-minute walk test (6MWT), over a placebo/delayed treatment cohort in our Phase IIb open label extension study. Eteplirsen administered once weekly at 50 mg/kg over 36 weeks resulted in a 69.4 meter benefit compared to patients who received placebo for 24 weeks followed by 12 weeks of treatment with eteplirsen. In the predefined prospective analysis of the study's intent-to-treat population on the primary clinical outcome measure, the change in 6MWT distance from baseline, eteplirsen-treated patients who received 50 mg/kg of the drug weekly demonstrated a decline of 8.7 meters in distance walked from baseline (mean=396.0 meters), while patients who received placebo/delayed-eteplirsen treatment for 36 weeks showed a decline of 78.0 meters from baseline (mean=394.5 meters), for a statistically significant treatment benefit of 69.4 meters over 36 weeks ($p \leq 0.019$). There was no statistically significant difference in the 6MWT between the cohort of patients who received 30 mg/kg weekly of eteplirsen and the placebo/delayed treatment cohort. The safety profile of eteplirsen was evaluated across all subjects through the 36 weeks and there were no treatment-related adverse events, no serious adverse events and no discontinuations. Furthermore, no treatment-related changes were detected on any safety laboratory parameters, including several biomarkers for renal function.

On October 3, 2012, we announced 48-week results from our DMD open label extension study which indicated that treatment with eteplirsen met the primary efficacy endpoint, increase in novel dystrophin, and achieved a significant clinical benefit on the primary clinical outcome, the 6MWT, over the placebo/delayed treatment cohort in our Phase IIb extension trial. Eteplirsen administered once weekly at either 30 mg/kg or 50 mg/kg for 48 weeks (n=8) resulted in a statistically significant increase ($p < 0.001$) in dystrophin-positive fibers to 47.0% of normal. The placebo/delayed treatment cohort, which had received 24 weeks of eteplirsen at either 30 mg/kg or 50 mg/kg following 24 weeks of placebo (n=4), also showed a statistically significant increase in dystrophin-positive fibers to 38.3% of normal ($p < 0.009$). Eteplirsen administered once weekly at 50 mg/kg over 48 weeks resulted in an 89.4 meter benefit compared to patients who received placebo for 24 weeks followed by 24 weeks of treatment with eteplirsen in the open-label extension. In the predefined prospective analysis of the study's intent-to-treat population on the primary clinical outcome measure, the change in 6MWT distance from baseline, eteplirsen-treated patients who received 50 mg/kg of the drug weekly (n=4) demonstrated an increase of 21.0 meters in distance walked from baseline (mean=396.0 meters), while patients who received placebo/delayed-eteplirsen treatment (n=4) showed a decline of 68.4 meters from baseline (mean=394.5 meters), for a statistically significant treatment benefit of 89.4 meters over 48 weeks ($p = 0.016$, using analysis of covariance for ranked data). There was no statistically significant difference between the cohort of patients who received 30 mg/kg weekly of eteplirsen and the placebo/delayed treatment cohort. The safety profile of eteplirsen was evaluated across all subjects through 48 weeks and there were no treatment-related adverse events, no serious adverse events, and no discontinuations. Furthermore, no clinically significant treatment-related changes were detected on any safety laboratory parameters, including several biomarkers for renal function. We anticipate initiating the enrollment of a pivotal Phase III trial in late 2013.

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We are also leveraging the capabilities of our RNA-based technology platforms to develop therapeutics for the treatment of infectious diseases. The U.S. Department of Defense, or DoD, has provided significant financial support for the development of therapeutics against Ebola, Marburg, and influenza viruses. As of September 30, 2012, we had completed all of our then-existing contracts with the U.S. government except for the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses (the ADHFVT contract). On August 29, 2012, we entered into an additional agreement with DoD related to the Marburg virus to evaluate the feasibility of an intramuscular route of administration using AVI-7288. On October 2, 2012, the Company received notice from DoD that the Ebola portion of the ADHFVT contract was terminated for the convenience of the government due to funding constraints. The Company previously received a stop-work order for the Ebola portion of the ADHFVT contract which was in effect from August 2, 2012 through the termination on October 2, 2012. The termination only applies to the Ebola portion of the ADHFVT contract and the Marburg portion remains in effect. For additional information, see Government Contracts below.

Since our inception in 1980, we have incurred losses of approximately \$369.2 million and substantially all of our revenue has been derived from research and development contracts with the U.S. government. We have not yet generated any material revenue from product sales and we have incurred expenses related to research and development, general and administrative charges and acquired in-process research and development resulting from two acquisitions. We expect to continue to incur losses in the future as we continue our research and development efforts and seek approval from various regulatory agencies for our product candidates, but there can be no assurance that we will obtain approval for our product candidates and achieve revenues from product sales.

As of September 30, 2012, we had cash and cash equivalents of \$38.0 million and in October 2012, we received \$16.4 million from the sale of stock and \$5.6 million from the exercise of previously outstanding warrants. Our principal sources of liquidity are equity financings and revenue from our U.S. government research contracts. We anticipate receiving continued funding from the U.S. government to pursue the development of our therapeutic against Marburg and are likely to pursue additional funding through public or private financings and cash generated from establishing collaborations or licensing our technology to other companies. Our principal uses of cash are research and development expenses, general and administrative expenses and other working capital requirements. Many of these uses of cash are discretionary in nature and can be significantly reduced at the discretion of management and our Board of Directors. The Company believes these sources of cash and potential reductions in discretionary spending, when combined together, would provide us with sufficient cash to fund operations at least through the following 12 months. Should our funding from the U.S. government cease or be further delayed, it would have a negative impact on our financial condition and we would significantly reduce our research and development efforts for therapeutics against the Marburg virus.

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

Government Contracts

We recognize revenues from U.S. government research contracts during the period in which the related expenditures are incurred and present these revenues and related expenses gross in the consolidated financial statements. In the periods presented, substantially all of the revenue generated by us was derived from research contracts with the U.S. government. As of September 30, 2012, we had completed all of our contracts with the U.S. government except for the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses and the contract for intramuscular injection (IM contract). On October 2, 2012, the U.S. government terminated the Ebola portion of the July 2010 agreement.

The following table sets forth the revenue for each of the contracts with the U.S. government for the three months and nine months ended September 30, 2012 and 2011.

	Three Months Ended		Nine Months Ended	
	September 30, 2012	September 30, 2011	September 30, 2012	September 30, 2011
	(in thousands)		(in thousands)	
July 2010 Agreement (<i>Ebola and Marburg</i>)	\$ 7,511	\$ 7,290	\$ 29,844	\$ 29,780
August 2012 Agreement (<i>Intramuscular</i>)	50		50	

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June 2010 Agreement (<i>H1N1</i>)		183		3,390
Other Agreements	13	51	99	235
Total	\$ 7,574	\$ 7,524	\$ 29,993	\$ 33,405

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July 2010 Agreement (Ebola and Marburg)

On July 14, 2010, we were awarded a contract with the U.S. Department of Defense, or DoD, Chemical and Biological Defense Program through the U.S. Army Space and Missile Defense Command 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. During 2012, we received permission from the FDA to proceed with single oligomers, AVI-7537 and AVI-7288, as the lead product candidates against the Ebola and Marburg virus infections, respectively and, in June 2012, requested a contract modification from DoD to proceed with these single oligomers as the lead product candidates against the Ebola and Marburg virus infections.

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 U.S. government terminated the Ebola portion of the contract for the convenience of the government due to recently imposed funding constraints. The Ebola portion of the contract with the DoD represented approximately half of the contract revenue. We anticipate a reduction in associated research and development costs as research on the Ebola virus therapeutic candidate will be substantially curtailed without further funding. These research and development costs are primarily with third party contractors. We anticipate that the impact to general and administrative expenses will be minimal.

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. After completion of the first segment, and each successive segment, DoD has the option to proceed to the next segment. Activities under the first segment began in July 2010 and include Phase I studies in healthy volunteers as well as preclinical studies which are scheduled to be completed in 2013. The remaining funding as of September 30, 2012 for the current Marburg segment is approximately \$19.5 million. If DoD exercises its options for segments II, III and IV for AVI-7288, contract activities would include all clinical and licensure activities necessary to obtain Food and Drug Administration (FDA) regulatory approval. The funding for segments II, III and IV of the Marburg virus portion of contract is estimated to be approximately \$84.4 million.

August 2012 Agreement (Intramuscular administration)

On August 29 2012, we were awarded a new contract from the U.S. Department of Defense's Joint Project Manager Transformational Medical Technologies (JPM-TMT) program, a component of the U.S. Department of Defense's Joint Program Executive Office for Chemical and Biological Defense. The contract provides to the Company of approximately \$3.9 million to evaluate the feasibility of an intramuscular (IM) route of administration using AVI-7288, our candidate for treatment of Marburg virus. The evaluation is scheduled to conclude in the second half of 2013. Under the July 2010 Agreement (Ebola and Marburg) described above, we are developing AVI-7288 as an intravenous formulation.

June 2010 Agreement (H1N1/Influenza)

On June 4, 2010, we entered into a contract with the Defense Threat Reduction Agency to advance the development of AVI-7100 as a medical countermeasure against the pandemic H1N1 influenza virus in cooperation with the Transformational Medical Technologies program of DoD. The period of performance for this contract ended on June 3, 2011.

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 6 U.S. Government Contracts of the unaudited financial statements included elsewhere in this report. We recognize revenue from U.S. government research contracts and grants during the period in which the related expenses are incurred and present such revenues and related expenses gross in the 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.

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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 upfront fees are deferred and recognized over the period of continuing involvement. As of September 30, 2012, we had deferred revenue of \$3.3 million, which represents upfront 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 on our ability to obtain U.S. government awards to fund the advanced development of our antiviral therapeutic candidates. Without such funding, we would likely drastically reduce our spending in these areas. Future research and development expenses may also increase if our internal projects, such as DMD, enter later stage clinical development. Our research and development programs are at an early stage 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 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 (Expense) and Other, Net. Interest income (expense) and other, net, consists of interest on our cash and cash equivalents, rental income and other income. Our cash equivalents consist of money market investments. Interest expense includes interest paid on our mortgage loan related to the Corvallis property. Other income includes rental income from subleasing excess space in some of our facilities.

Change in Fair Value of Warrants. Warrants issued in connection with our December 2007 and January and August 2009 financings are classified as liabilities, as opposed to equity, due to their settlement terms which require settlement in registered shares. These warrants are non-cash liabilities and 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. The fair value of the warrants is determined using the Black-Scholes 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 Warrants of the unaudited financial statements included elsewhere in this report.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are based upon our unaudited financial statements included elsewhere in this report. The preparation of our financial statements in accordance with accounting

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principles generally accepted in the United States, or GAAP, requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues 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.

Our critical accounting policies and significant estimates are detailed in our annual report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on March 13, 2012.

Results of Operations for the Three and Nine Months Ended September 30, 2012 and 2011

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

	Three Months Ended September 30, 2012		2011	% Change	Nine Months Ended September 30, 2012		2011	% Change
	(in thousands, except per share amounts)				(in thousands, except per share amounts)			
Revenue:	\$ 7,574	\$ 7,524		1%	\$ 29,993	\$ 33,405		(10)%
Expenses:								
Research and development	10,914	15,610		(30)%	39,568	48,161		(18)%
General and administrative	3,565	3,185		12%	9,761	12,171		(20)%
Operating loss	(6,905)	(11,271)		(39)%	(19,336)	(26,927)		(28)%
Other income (loss):								
Interest (expense) income and other, net	67	199		(66)%	270	440		(39)%
Gain (loss) on change in warrant valuation	(42,716)	7,052		(706)%	(40,154)	25,579		(257)%
Net income (loss)	\$ (49,554)	\$ (4,020)		1133%	\$ (59,220)	\$ (908)		6422%
Basic income (loss) per share	\$ (2.17)	\$ (0.18)			\$ (2.61)	\$ (.04)		
Diluted income (loss) per share	\$ (2.17)	\$ (0.18)			\$ (2.61)	\$ (.04)		

Revenue

Revenue for the three months ended September 30, 2012 increased by approximately \$0.1 million, or 1%, compared to the three months ended September 30, 2011. The increase was due to a \$1.1 million increase in revenue associated with the Marburg portion of the U.S. government research contract and a \$0.9 million decrease in the Ebola portion of the U.S. government contract due primarily to the stop-work-order we received on August 2, 2012. Additionally, on October 2, 2012, we received a termination letter for the Ebola portion of the contract due to U.S.

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government funding constraints. The termination of the Ebola portion of the contract does not impact the Company's ongoing Marburg activities. Ebola portion of the contract with the DoD represented approximately half of the contract revenue.

Revenue for the nine months ended September 30, 2012 decreased by \$3.4 million, or 10%, compared to the nine months ended September 30, 2011. The decrease in revenue was due primarily to a \$3.4 million decrease in the H1N1 U.S. government research contracts which were substantially completed in 2011.

Research and Development Expenses

Research and development expenses for the three months ended September 30, 2012 decreased by \$4.7 million, or 30%, compared to the three months ended September 30, 2011. The decrease was primarily due to a \$4.0 million reduction in personnel related costs and costs of proprietary research, a \$1.3 million decrease in our DMD program costs due to the timing

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of manufacturing and clinical activities and a decrease of \$0.3 million from the Ebola portion of the government contract due to the August 2012 stop work order. The decrease was partially offset by higher costs associated with the Marburg portion of the government research contract of \$0.4 million.

Research and development expenses for the nine months ended September 30, 2012 decreased by \$8.6 million, or 18%, compared to the nine months ended September 30, 2011. The decrease was primarily due to a \$5.6 million decrease in personnel related costs and costs related to our proprietary research due to the December 2011 restructuring, a \$2.6 million decrease in spending related to the H1N1 U.S. government research contracts which were concluded in June of 2011, a decrease of \$2.1 million of the Ebola portion of the government contract, and a \$0.2 million decrease in our DMD related costs. The decrease was offset by higher cost in our Marburg portion of the government contract totaling \$1.9 million.

General and Administrative Expenses

General and administrative expenses for the three months ended September 30, 2012 increased by \$0.4 million, or 12%, compared to the three months ended September 30, 2011. The increase in general and administrative expense is primarily due to increased legal costs.

General and administrative expenses for the nine months ended September 30, 2012 decreased by \$2.4 million, or 20%, compared to the nine months ended September 30, 2011. The decrease is primarily due to a \$2.0 million decrease in salaries, severance, and other employee related costs, \$0.5 million decrease in professional services costs and a \$0.3 million reduction in facilities costs. The decrease was partially offset by higher legal fees of \$0.4 million.

Interest (Expense) Income and Other, Net

Interest income (expense) and other, net, for the three and nine months ended September 30, 2012 decreased due to lower interest income earned on reduced cash and cash equivalents balances compared to the three and nine months ended September 30, 2011.

Change in Fair Value of Warrant Liability

The changes in fair value of warrant liability for the three and nine months ended September 30, 2012 compared to the three month and nine months ended September 30, 2011 was primarily attributable to changes in our stock price. See Key Financial Metrics Change in Fair Value of Warrants, and Note 5 to the unaudited condensed consolidated financial statements included elsewhere in this report.

Net Income (Loss)

Net loss for the three months ended September 30, 2012 was \$49.6 million, compared to net loss of \$4.0 million for the three months ended September 30, 2011. The increased net loss was primarily due to \$49.8 million increase in nonoperating expenses due to the increase in the fair market value of our outstanding warrants. The fair market value of our outstanding warrants is a noncash expense which was highly impacted based on the increase in our stock price. This increase was partially offset by a reduction in our operating loss of \$4.4 million.

Net loss for the nine months ended September 30, 2012 was \$59.2 million, compared to the net loss of \$0.9 million for the nine months ended September 30, 2011. The increase was primarily due to the increase in our warrant liability by \$65.7 million partially offset by reduced operating loss of \$7.6 million.

Liquidity and Capital Resources

At September 30, 2012, cash and cash equivalents were \$38.0 million, compared to \$39.9 million at December 31, 2011. In addition, during October 2012, we received \$16.4 million from the sale of stock and \$5.6 million from the exercise of previously outstanding warrants. Our principal sources of liquidity are equity financings and revenue from our U.S. government research contracts. Our principal uses of cash are research and development expenses, general and administrative expenses and other working capital requirements. Many of these uses of cash are discretionary in nature and can be significantly reduced at the discretion of our management and the Board of Directors. The Company believes these sources of cash and potential reductions in discretionary spending, when combined together, would provide sufficient cash to fund our operations for at least the following 12 months. Should our funding from the U.S. government cease or be delayed, it would have a negative impact on our financial condition and we would significantly reduce research and development efforts for therapeutics against the Marburg virus.

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Our primary source of revenue is from development of product candidates pursuant to our contracts with the U.S. government. 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. If U.S. government funding is not received or is further delayed, our results of operations would be materially and adversely affected and we may need to seek additional sources of capital and significantly curtail our current operations. We do not generate any revenue from non-government, commercial sale of our pharmaceutical product candidates.

In April 2011, we sold approximately 3.8 million shares (as adjusted for the effect of our July 2012 one-for-six reverse stock split) of our common stock at \$9.00 per share (as adjusted for the effect of our July 2012 one-for-six reverse stock split) in an offering registered under the Securities Act of 1933, or the Securities Act. The offering generated net proceeds of approximately \$32.1 million.

In September 2012 we sold 1.4 million shares under our equity financing arrangement and received net proceeds of \$19.9 million. During the same period we received proceeds totaling \$0.3 million from the exercise of 80,014 warrants. Subsequent to September 30, 2012, and through October 31, 2012, we sold 0.6 million shares under our equity financing arrangement and received net proceeds of \$16.4 million and we received proceeds totaling \$5.6 million from the exercise of 0.5 million warrants. In addition, as of October 31, 2012, we have warrants to purchase 709,130 shares of common stock, which expire on December 18, 2012. These warrants have an exercise price of \$14.70 and if they are exercised, we will receive an additional \$10.4 million in proceeds.

We will require additional capital from time to time in order to fund our operations, continue the development of products and to expand our product portfolio. We expect to seek additional financing primarily from, but not limited to, the sale and issuance of equity or debt securities. In addition, we may license portions of our proprietary technologies. We cannot assure you that financing or partnering opportunities will be available when and as needed or that, if available, they will be on favorable or acceptable terms. If we are unable to obtain additional sources of funds when and if we require, it would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing shareholders could experience substantial dilution.

We have never generated revenue from the sale of commercial products and cannot offer any assurances that we will be able to do so in the future.

Uses of Funds

From inception in 1980 through September 30, 2012, our accumulated deficit is \$369.2 million. Our principal uses of cash have been research and development expenses, general and administrative expenses, acquired in-process research and development resulting from two acquisitions, costs associated with the acquisition of in-process research and development and other working capital requirements.

Historical Trends

	Nine Months Ended September 30,	
	2012	2011
	(in thousands)	
Cash provided by (used in):		
Operating activities	\$ (22,536)	\$ (18,031)
Investing activities	(722)	(1,521)
Financing activities	21,341	32,319
Increase (decrease) in cash and equivalents	\$ (37,987)	\$ 46,356

Operating Activities. We used \$22.5 million of cash in operating activities for the nine months ended September 30, 2012, compared to \$18.0 million of cash used in operating activities for the nine months ended September 30, 2011. The increase in net cash used in operations during the comparative periods was primarily attributable to an \$11.5 million decrease in cash provided from changes in working capital and partially offset by a \$7.4 million decrease in net loss, excluding the noncash loss associated with the periodic revaluation of our warrants to fair market value.

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Investing Activities. We used \$0.7 million of cash in investing activities for the nine months ended September 30, 2012, compared to the \$1.5 million of cash used in investing activities for the nine months ended September 30, 2011. Less cash was used for the purchase of property and equipment costs in the nine months ended September 30, 2012, compared to 2011.

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Financing Activities. Cash provided by financing activities for the nine months ended September 30, 2012 was attributable to September 2012 equity financing generating \$19.9 million in net proceeds and exercise of stock option and warrants generating \$1.5 million in September 2012. Cash provided by financing activities for the nine months ended September 30, 2011 were primarily due to the April 2011 equity financing that generated net proceeds of \$32.1 million.

Our future expenditures and capital requirements depend on numerous factors, most of which are difficult to project beyond the short term. These requirements include our ability to meet the requirements of our U.S. government research projects, the government's ability to fund such projects, the progress of our research and development programs and our pre-clinical and clinical trials, 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 commercialization of our products. We anticipate we will need additional cash as we continue to advance our research, development and commercialization programs.

Contractual Obligations and Contingencies

In our continuing operations, we have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and acquisition of technology access rights, among others. The following table presents noncancelable contractual obligations arising from these arrangements as of September 30, 2012:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years (in thousands)	3-5 Years	More Than 5 Years
Long-term debt	\$ 1,778	\$ 88	\$ 188	\$ 208	\$ 1,294
Operating leases (1)	13,244	2,150	4,286	4,597	2,211
Purchase obligations (2)	637	637			
Total	\$ 15,659	\$ 2,875	\$ 4,474	\$ 4,805	\$ 3,505

- (1) In May 2012, the Company exercised its option to terminate a lease for its laboratory and administrative office facility in Bothell, Washington effective May 2013.
- (2) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding to the Company 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 10 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.**Interest Rate Sensitivity**

We had cash and cash equivalents of \$38.0 million and \$39.9 million at September 30, 2012 and December 31, 2011, respectively. We do not enter into investments for trading or speculative purposes and 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 extremely low rates of investment interest and to the short term nature of our cash and cash equivalents. Future declines in interest rates, however, would reduce investment income, but

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are not likely to be a material source of revenue to our company in the foreseeable future. A 0.1% decline in interest rates, occurring January 1, 2012 and sustained throughout the period ended September 30, 2012, would be inconsequential.

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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 principal financial officer and (2) our principal accounting 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 principal financial officer and our principal accounting officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2012, 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, 2012 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. It is impossible to predict whether any resulting liability would have a material adverse effect on our 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 and AVI-7288 in Marburg are in active clinical development. AVI-7537 in Ebola was in active clinical development until August 2012, when we received a stop-work order from DoD instructing us to cease all work and ordering of supplies in support of the development of this product candidate. On October 2, 2012, we received notice from DoD that the program for the development of AVI-7537 was terminated for the convenience of the government due to funding constraints. The clinical development of AVI-7100 in influenza is currently paused and 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 and our antiviral candidates. With current resources, we may be restricted or delayed in our ability to develop other clinical and preclinical product candidates.

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Our ability to commercialize any of our product candidates, including eteplirsen, depends on first receiving required regulatory approvals, and it is possible that we may never receive regulatory approval (including any accelerated approval by the U.S. Food and Drug Administration (the FDA) under Subpart H Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses) for any of our product candidates based on an inability to adequately demonstrate the safety and effectiveness of our product candidates, lack of funding, changes in the regulatory landscape, manufacturing or other reasons. 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 at acceptable costs;

marketing and distribution support for the product; and

any exclusivities applicable to the product.

To date we have been granted orphan status for two of our product candidates in DMD and for AVI-6002 and AVI-7537 for the treatment Ebola virus and AVI-6003 and AVI-7288 for the treatment of Marburg virus. We are not guaranteed to receive orphan status for other product candidates in development or product candidates we may develop in the future. Even though we have received orphan status for some of our product candidates, we would not enjoy orphan drug exclusivity for such product candidates in the event that another entity received approval of products with the same active ingredient for the same indication before we receive market approval. 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 a competitor's product receives orphan drug status for an indication that we are targeting, and such product is approved for commercial sales before our product, regulators may interpret our product to be the same drug as the competing product and could prevent us from selling our product in the applicable territories for the competitor's orphan exclusivity period. Furthermore, pediatric exclusivity only applies if the product has another form of exclusivity.

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.

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, selling, marketing and distribution of drug products are subject to extensive regulation by 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 marketing approval from the FDA or other foreign regulatory authorities, and we may never receive regulatory approval for the commercial sale of any of our product candidates. Obtaining marketing approval is a lengthy, expensive and uncertain process and approval is never assured. As of the date of this report, we have not progressed to the point of preparing or filing the applications necessary to gain regulatory approvals. Further, the FDA and other foreign regulatory agencies have substantial discretion in the approval process, and determining when or whether regulatory approval will be obtained

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for any product candidate we develop. In this regard, even if we believe the data collected from clinical trials of our product candidates are promising, such data may not be sufficient to support approval by the FDA or any other foreign regulatory authority. In addition, the FDA or their advisors may disagree with our interpretations of data from preclinical

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studies and clinical trials. Regulatory agencies may approve a product candidate for fewer indications than requested or may grant approval subject to the performance of post-approval studies for a product candidate. Similarly, 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 regulatory requirements and guidance may occur and we may need to amend clinical trial protocols or other approval strategies to reflect these changes. 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. Changes in our approval strategies may require additional studies that were not originally planned. Other factors may also impact our ability to commercialize our product candidates, including, for example, the fact that a therapeutic commercial product utilizing our RNA-based technologies has never been approved by any regulatory authority. Due to these factors, 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 product revenue by delaying or terminating the potential commercialization of our product candidates.

If we receive regulatory approval for our product candidates, we will also be subject to ongoing FDA obligations and oversight, including adverse event reporting requirements, marketing restrictions and, potentially, other post-marketing obligations, all of which may result in significant expense and limit our ability to commercialize such products. The FDA's policies may also change and additional government regulations may be enacted that could prevent or delay regulatory approval of our product candidates or 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 need to obtain regulatory approval from authorities in foreign countries to market our product candidates in those countries. We have not filed 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.

Phase I clinical trials generally are not designed to test the efficacy of a product candidate but rather are designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the product candidate's side effects at various doses and dosing schedules in healthy volunteers. Delays in establishing the appropriate dosage levels can lead to delays in the overall clinical development of a product candidate. As of the date of this report, we do not believe that we have identified the preferred dose of eteplirsen for individuals with DMD. We plan to evaluate the appropriate dosage in a future confirmatory pivotal study. We recently completed a U.S.-based Phase IIb clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg, higher doses than was initiated in August 2011. Following completion of this study, we initiated an open label extension study with the same participants from the original Phase IIb placebo controlled trial. These trials were initiated, in part, to further explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. We cannot assure you that these efforts will be successful. If a consistently effective dose is found in the U.S.-based clinical trial, we will expect to engage in discussions with regulatory authorities about the design and subsequent execution of any further studies which may be required. Regulatory authorities might require more extensive preclinical or clinical trials than anticipated and conforming to any guidance regulatory authorities provide does not guarantee receipt of marketing approval, even if we believe our preclinical and clinical trials are successful. 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) and any additional placebo-controlled pivotal study or studies. If we are not able to establish an optimal dosage in these trials we may need to conduct additional dose-ranging trials before conducting our pivotal trials of the product. Any such additional clinical trials required by regulatory authorities would increase our costs and delay commercialization of eteplirsen.

Furthermore, success in preclinical and early clinical trials does not ensure that later larger-scale trials will be successful nor does it predict final results. Acceptable results in early trials may not be reproduced in later trials. For example, pivotal trials for eteplirsen will likely involve a larger number of participants to achieve statistical significance, will be expensive and

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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.

The Animal Rule is a new and seldom-used approach to seeking approval of a new drug and our infectious disease program may not meet the requirements for this ill-defined 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 in humans as it relates to efficacy 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. No novel products have been approved using the Animal Rule. It has thus far been used to extend the indicated use of three previously licensed 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 rely on U.S. government contracts to support certain research and development programs and 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 would be materially and adversely affected.

We rely on U.S. government contracts and awards to fund certain development programs, including the Marburg virus therapeutic candidate 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. Additionally, the DoD is planning on hundreds of billions of dollars in cuts to defense spending over the next decade and faces a possible sequestration of an additional \$600 billion over the same timeframe beginning in January 2013 unless Congress acts. These cuts would have widespread ramifications including on DoD's procurement and research and development programs. The 2004 Project BioShield Act which created the Special Reserve Fund for use by DHHS to purchase countermeasures over 10 years avoids the uncertainty of the annual appropriations process, but the \$5.6 billion appropriation is rapidly depleting and will expire in 2013. Thus, the viability of DHHS as a 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 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

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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 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 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 product candidate, 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 drug candidate will successfully meet these requirements. If it does not, DoD may choose to terminate the contract. With respect to the civilian sector, Marburg virus is among the top chemical, biological, radiological and nuclear threats to national security, yet DHHS has not defined the civilian requirement, making the broader demand for our drug candidate 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 U.S. government 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.

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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 U.S. government. Negotiating and entering into such arrangements can be time-consuming and we may not be able to reach agreement with such third parties. Any such agreement also has to be compliant with the terms of our government grants. 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 U.S. government.

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. We expect to commence additional trials of eteplirsen and other product candidates in the future. 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 scheduling conflicts with participating clinicians and clinical institutions, difficulties in identifying and enrolling participants who meet trial eligibility criteria, failure of participants to complete the clinical trial, delay or failure to obtain IRB or other regulatory approval to conduct a clinical trial at a prospective site, unexpected adverse events and shortages of available drug supply. 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, or CROs, to conduct our clinical trials in compliance with Good Clinical Practice, or 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 (e.g., 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. In addition, clinical trials must be conducted with supplies of our product candidates produced under current Good Manufacturing Practice, or cGMP, and other requirements in foreign countries, and may require large numbers of participants. 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:

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;

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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;

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the time required to determine whether the product candidate is effective may be 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 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 drug 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 delay of the program. Negative 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 safety monitoring board, or DSMB, and the 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.

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

We had an operating loss of \$19.3 million for the nine months ended September 30, 2012, and incurred an operating loss of \$35.9 million for the year ended December 31, 2011. As of September 30, 2012, our accumulated deficit was \$369.2 million. Our operating losses have resulted principally from expenses incurred in research and development of our technology and products, from general and administrative expenses that

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we have incurred while building our business infrastructure and acquired in-process research and development resulting from two acquisitions. We expect to continue to incur significant operating losses in the future as we continue our research and development efforts and seek to obtain regulatory approval of our products. Our ability to achieve profitability depends on our ability to raise additional capital, partner one or more programs, complete development of our products, obtain regulatory approvals and market our products. It is uncertain when, if ever, we will become profitable.

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

We will 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 will need will be determined by many factors, some of which are beyond our control. These factors include the success of our research and

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development efforts, the status of our preclinical and clinical testing, costs relating to securing regulatory approvals and the costs and timing of obtaining new patent rights, regulatory changes and competitive and technological developments in the market. 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 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 shareholders. To the extent we issue additional equity securities, our existing shareholders could experience substantial dilution in their economic and voting rights. For example, through November 7, 2012, we sold an aggregate of approximately 14.0 million shares (which number gives effect to our July 2012 one-for-six reverse stock split) of our common stock in connection with our December 2007, January 2009, August 2009 and April 2011 financings and September 2012 at-the-market equity offering program and issued warrants to purchase approximately 5.0 million additional shares (which number gives effect to our July 2012 one-for-six reverse stock split) of our common stock in connection with our December 2007, January 2009 and August 2009 financings, which warrants have been exercised for an aggregate of 0.7 million shares of our common stock.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our RNA-based technologies, research programs or to conduct clinical trials and to market our product candidates. Other than pre-clinical collaborations with academic/research institutions and a U.S. government entity for the development of additional exon-skipping drug candidates for the treatment of DMD, we currently do not have a strategic relationship with a third party to perform research or development using our RNA-based technologies or assist us in funding the continued development and commercialization of any of our programs or drug candidates other than that with the U.S. government. If we are unable to enter into partnerships or strategic relationships with respect to our technologies or any of our programs or drug candidates on favorable terms it may impede our ability to discover, develop and commercialize our product candidates.

We currently rely on third-party manufacturers and other third parties for production of our drug products 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 the product candidates 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 substance. We may 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, fill vials and store sufficient quantities of our product candidates for use in our research and development programs and clinical trials. For each of our eteplirsen and Marburg development programs, based on limited capacity for our specialized manufacturing needs we have had to enter into a sole-source agreement with multinational manufacturing firms for the production of the API for eteplirsen and Marburg therapeutics. There are a limited number of companies that can produce phosphorodiamidate-linked morpholino oligomer, or PMO, in the quantities and with the quality and purity that we require for our development efforts. This might limit our ability to rapidly expand our programs or commercialize our products. If we are required to seek alternative supply arrangements, the resulting delays and potential inability to find suitable replacements or bring on-line new suppliers 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 and for clinical use 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 or otherwise discontinue development and production of our product candidates. In addition, we depend on certain sole-source third-party vendors 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 quality raw materials and we are unable to contract on acceptable terms for these raw materials with alternative suppliers, our ability to have our product candidates manufactured and to conduct preclinical testing and clinical trials of our product candidates would be adversely affected.

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We do not yet have all of the agreements necessary for the supply of our product candidates in quantities sufficient for commercial sale 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 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 operation of those facilities due to events such as 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.

Our contract manufacturers are required to produce our clinical product candidates under 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 obligations under 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, cause us to lose revenue, 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 product candidates, 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. In order to conduct larger or late-stage scale clinical trials for a product candidate and for commercialization of the resulting drug product 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 validation studies, which are costly 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 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.

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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 therapeutic 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 internal 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.

The relocation of our corporate headquarters and selected research and development activities may create unintended negative consequences, including increased costs and loss of personnel.

We plan to move our corporate headquarters from Bothell, Washington to Cambridge, Massachusetts and move selected research and development activities from Bothell to our existing site in Corvallis, Oregon and a yet to be selected site in Cambridge. This transition is in the early planning stage and we expect the transition will continue through mid-2013. While we believe the relocation will improve our business operations and enhance our ability to attract and retain industry talent in the Cambridge area, we cannot ensure that this relocation will not result in any or all of the following unintended negative consequences:

increased costs associated with the closing of our existing facility in Bothell, Washington including the moving of lab equipment and furniture to Cambridge and Corvallis;

increased costs associated with the relocation of personnel, including reimbursement of relocation expenses and cost of living adjustments to base salaries;

employee turnover due to relocation;

increased costs associated with retention and/or severance packages for Bothell based personnel;

business disruptions resulting from the relocation; and

inability to locate suitable administrative and laboratory space for a long-term lease arrangement in Cambridge at a reasonable cost. If any of these unintended negative consequences occurs, the negative impact may outweigh any benefits related to the relocation, which could have an adverse effect on our business.

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.

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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.

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Recent changes in our executive leadership and any similar changes in the future may serve as a significant distraction for our management and employees.

In January 2011, Christopher Garabedian, a member of our board of directors, was hired to serve as our president and chief executive officer. Since the beginning of 2011, there have been a number of changes to our executive leadership team. Most recently, in June 2012, our former senior vice president and chief scientific officer, Dr. Peter Linsley, resigned from his employment with us. Such changes, or any other future changes in our executive leadership, may disrupt our operations as we adjust to the reallocation of responsibilities and assimilate new leadership and, potentially, differing perspectives on our strategic direction. If the transition in executive leadership is not smooth, the resulting disruption could negatively affect our operations and impede our ability to execute our strategic plan.

We may engage in future acquisitions that increase our capital requirements, dilute our shareholders, 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. Any potential acquisitions 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.

Asserting, defending and maintaining our intellectual property rights could be challenging and costly, and our failure to do so could harm our ability to compete and impair the outcome of our operations. The pharmaceutical, biotechnology and academic environments are highly competitive and competing intellectual property could limit our ability to protect our products.

Our success will depend in significant part on our existing intellectual property rights and our ability to obtain additional patents and licenses in the future. As of October 31, 2012, we owned or controlled approximately 280 U.S. and corresponding foreign patents and 180 U.S. and corresponding foreign patent applications. We license patents from other parties for certain complementary technologies. We cannot be certain that pending patent applications will result in patents being issued in the United States or foreign countries. We cannot be certain that we were the first to make the inventions covered by any of our patents, if issued, or our pending patent applications. In addition, the patents that have been or will be issued may not afford meaningful protection for our technology and products. Competitors may develop products similar to ours that do not conflict with our patents. To protect our rights to any of our patents, if issued, and proprietary information, we may need to litigate against infringing third parties, or avail ourselves of the courts or participate in hearings to determine the scope and validity of those patents or other proprietary rights. These types of proceedings are often costly and could be very time-consuming to us, and we cannot assure you that the deciding authorities will rule in our favor. An unfavorable decision could allow third parties to use our technology without being required to pay us licensing fees or may compel us to license needed technologies to avoid infringing third-party patent and proprietary rights.

Pharmaceutical research and development is highly competitive; others may file patents first that cover our products or technology. For example, our competitor Prosensa has rights to patent families corresponding to WO2002/024906 and WO2004/083432, including issued US 7,973,015, US 7,534,879, and granted European Patent No. EP 1619249. We opposed EP 1619249 in the Opposition Division of the European Patent Office, or the Opposition Division, and in November 2011, we announced that, although we succeeded in invalidating some of the patent's claims, the Opposition Division maintained in amended form certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46. We and Prosensa both have the right to appeal this decision; however, pending final resolution of this matter and any appeal thereof, the patent at issue may provide the basis for Prosensa or other parties that have rights to such patent to assert that our drug eteplirsén infringes on such patent. A final resolution of this opposition proceeding may take a number of years and the outcome cannot be predicted or determined as of the date of this report. We are also aware of certain claims that have issued to Prosensa in Japan (JP 4846965) that may provide the basis for Prosensa or other parties that have rights to these claims to assert that our drug eteplirsén infringes on such claims. We believe we have a basis to invalidate some or all of these claims and are evaluating the potential initiation of invalidation proceedings. Because we have not yet initiated an invalidation proceeding in Japan, the outcome and timing of such proceeding cannot be predicted or determined as of the date of this report. If we are unsuccessful in invalidating other of Prosensa's claims or if previously invalidated claims are restored on appeal, our ability to commercialize both eteplirsén and other therapeutic candidates for our pan-exon strategy could be materially impaired.

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Our success will also depend partly on our ability to operate without infringing upon the proprietary rights of others as well as our ability to prevent others from infringing on our proprietary rights. We may be required at times to take legal action to protect our proprietary rights and, despite our best efforts, we may be sued for infringing on the patent rights of others. We have not received any communications or other indications from owners of related patents or others that such persons believe our products or technology may infringe on their patents. Patent litigation can involve complex factual and legal questions and its outcome is uncertain. Patent litigation is costly and, even if we prevail, the cost of such litigation could adversely affect our financial condition. If we do not prevail, in addition to any damages we might have to pay, we could be required to stop the infringing activity or obtain a license. If any patent related to our products or technology issues, and if our activities are determined to be covered by such a patent, we cannot assure you that we will be able to obtain or maintain a license, which could have a material adverse effect on our business, financial condition, ability to sell our products, operating results and ability to obtain and/or maintain our strategic business relationships.

Others may challenge our patents and, as a result, our patents could be narrowed or invalidated. The patent position of pharmaceutical and biotechnology firms, as well as academia, is generally highly uncertain, involves complex legal and factual questions, and has recently been the subject of much litigation. No consistent policy has emerged from the U.S. Patent and Trademark Office, or USPTO, or the courts regarding the breadth of claims allowed or the degree of protection afforded under biotechnology patents. In addition, there is a substantial backlog of pharmaceutical and biotechnology patent applications at the USPTO and the approval or rejection of patents may take several years.

To help protect our proprietary rights in unpatented proprietary information, trade secrets and know-how, we require our employees, consultants and advisors to execute confidentiality agreements and invention assignment agreements. However, such agreements may not provide us with adequate protection if confidential information is used or disclosed improperly. In addition, in some situations these agreements may conflict with, or be subject to, the rights of third parties with whom our employees, consultants or advisors have prior employment or consulting relationships. Further, others may independently develop substantially equivalent proprietary information and techniques, or otherwise gain access to our trade secrets.

Our research collaborators may publish data and information to which we have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations, then our ability to receive patent protection or protect our proprietary information may be impaired.

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 share a focus on RNA-based drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include Prosensa and GlaxoSmithKline, or GSK, and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs.

Clinical trials evaluating the systemic administration of the Prosensa/GSK lead DMD drug candidate are currently ongoing, including a placebo-controlled global Phase III trial and two placebo-controlled Phase II trials, one based in the United States and one based outside the United States. The Prosensa/GSK drug candidate may, or may not, prove to be safer or more efficacious than our product candidate and it could gain marketing approval before our product candidate. This might affect our ability to successfully complete a clinical development program or market eteplirsen once approved. This competition may also extend to other exon-skipping drugs for DMD limiting our ability to gain market share.

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;

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develop less costly products;

obtain quicker regulatory approval;

have access to more manufacturing capacity;

develop products that are more convenient and easier to administer;

form more advantageous strategic alliances; or

establish superior proprietary 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 corporate 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 corporate collaborators or others selling such products. We may experience financial losses in the future due to 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.

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Risks Related to Our Common Stock

Our recently completed one-for-six reverse stock split could adversely impact our company and the trading of our common stock.

On July 11, 2012, we filed an amendment to our Fourth Restated and Amended Articles of Incorporation with the Secretary of State of the State of Oregon to, in part, effect a one-for-six reverse stock split. While the reverse stock split enabled us to regain compliance with The NASDAQ Stock Market's minimum bid price listing requirement, it may also result in certain adverse impacts to our company and the trading of our common stock. The reverse stock split may be viewed negatively by the market and, consequently, could lead to a decrease in our price per share and overall market capitalization. Additionally, the liquidity of our common stock could be adversely affected by the reduced number of shares resulting from the reverse stock split, which, in turn, could result in greater volatility in the price per share.

Our common stock may become ineligible for listing on The NASDAQ Stock Market, which would materially and adversely affect the liquidity and price of our common stock.

Our common stock is listed on The NASDAQ Global Market. The NASDAQ Global Market has several quantitative and qualitative requirements with which companies must comply in order to maintain this listing, including a \$1.00 minimum bid price per share and \$50 million minimum value of listed securities. On December 13, 2011, we received a letter from the listing qualifications department staff of The NASDAQ Stock Market, notifying us that for the previous 30 consecutive business days the bid price of our common stock had closed below \$1.00 per share, the minimum closing bid price required by the continued listing requirements of NASDAQ set forth in Listing Rule 5450(a)(1). On February 21, 2012, we received a letter from the listing qualifications department staff of The NASDAQ Stock Market that our common stock's closing bid price had been at or above \$1.00 per share for 10 consecutive business days and, thus, we had regained compliance with Listing Rule 5450(a)(1). On May 31, 2012, we received another letter from the listing qualifications department staff of The NASDAQ Stock Market, notifying us that we were not in compliance with the minimum bid price listing requirement. On July 11, 2012, we filed an amendment to our Fourth Restated and Amended Articles of Incorporation with the Secretary of State of the State of Oregon to, in part, effect a one-for-six reverse stock split. Following the completion of the reverse stock split we regained compliance with the minimum bid price listing requirement. Although we have regained compliance with this listing rule, we could in the future be unable to meet The NASDAQ Global Market continued listing requirements. If we fail to maintain compliance with The NASDAQ Stock Market's listing standards, and our common stock becomes ineligible for listing on The NASDAQ Stock Market the liquidity and price of our common stock would be adversely affected.

If our common stock was delisted, the price of our stock and the ability of our shareholders to trade in our stock would be adversely affected. In addition, we would be subject to a number of restrictions regarding the registration of our stock under U.S. federal securities laws, and we would not be able to allow our employees to exercise their outstanding options, which could adversely affect our business and results of operations. If we are delisted in the future from The NASDAQ Stock Market, there may be other negative implications, including the potential loss of confidence by actual or potential collaboration partners, suppliers and employees and the loss of institutional investor interest in our company.

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. 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:

positive or negative results of testing and clinical trials by ourselves, strategic partners, or competitors;

delays in entering 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;

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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;

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the perception that shares of our common stock may be delisted from The NASDAQ Stock Market; or

general market conditions in our industry or in the economy as a whole.

In addition, the stock market has recently experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of individual companies. 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 instigated against these companies. Such litigation, if instigated against us, could result in substantial costs and a diversion of our management's attention and resources.

Provisions of our articles of incorporation, bylaws and Oregon corporate 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 articles 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:

classification of our board of directors into two classes, with one class elected each year;

prohibition of cumulative voting of shares in the election of directors;

prohibition of shareholder actions by less than unanimous written consent;

express authorization of the board of directors to make, alter or repeal our bylaws;

advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by shareholders at shareholder meetings; and

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 shareholder approval, including rights superior to the rights of the holders of common stock.

In addition, the Oregon Control Share Act and Business Combination Act may limit parties that acquire a significant amount of voting shares from exercising control over us for specific periods of time. These provisions could discourage, delay or prevent a transaction involving a change of control, even if doing so would benefit our shareholders. These provisions also could discourage proxy contests and make it more difficult for shareholders to elect directors of their choosing or cause us to take other corporate actions, such as replacing or removing management or members of our 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 more pronounced than they were in the past as a result of the issuance of warrants to purchase approximately 5.0 million shares (which number gives effect to our July 2012 one-for-six reverse stock split) of our common stock by us in December 2007 and January and August 2009. Each of these warrants is classified as a derivative liability. Accordingly, the fair value of the warrants is recorded on our 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 consolidated statement of operations. The fair value of the warrants is determined using the Black-Scholes option valuation model. Fluctuations in the assumptions and factors used in the Black-Scholes model can result in adjustments to the fair value of the warrants reflected on our balance sheet 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.

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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 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. In addition, the market price of our common stock may fluctuate or decline regardless of our operating performance.

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

As of September 30, 2012, there were 24,302,261 shares of common stock outstanding, outstanding options to purchase 2,440,470 shares of common stock, restricted stock units representing 38,911 shares, stock appreciation rights representing 70,000 shares and outstanding warrants to purchase 4,822,090 shares of common stock. Additionally, as of September 30, 2012, there were 1,539,930 shares of common stock available for future issuance under our 2011 Equity Incentive 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 2011 Equity Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock or exercise of outstanding warrants or options will have a dilutive impact on other shareholders 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.

On September 4, 2012, we issued 1,576 shares of our common stock in an unregistered sale of common stock in connection with the exercise of an outstanding warrant originally issued and sold in January 2009. This warrant was exercised pursuant to a cashless exercise provision whereby the aggregate number of shares issued upon the exercise of the warrant was equivalent to the quotient obtained by dividing (i) the product of the number of shares underlying the warrant and the difference between the closing sale price immediately preceding the date of exercise, or the Closing Sale Price, and the exercise price, by (ii) the Closing Sale Price. The exercise price at the time of exercise was \$8.70 per share.

On September 11, 2012, we issued 39,704 shares of our common stock in an unregistered sale of common stock in connection with the exercise of an outstanding warrant having an exercise price of \$1.0074 per share and resulting in aggregate proceeds of approximately \$40,000. This warrant was issued in connection with our previous acquisition of Ercole Biotech, Inc. in March 2008.

The shares of common stock described above were issued without registration under the Securities Act, in reliance upon an exemption from registration under Section 4(a)(2).

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				
		Form	File No.	Exhibit	Filing Date	Filed Herewith
3.1	Fourth Restated and Amended Articles of Incorporation.	10-Q	001-14895	3.1	8/7/12	
3.2	Amendment to Fourth Restated and Amended Articles of Incorporation.	10-Q	001-14895	3.2	8/7/12	
3.3	Amended and Restated Bylaws.	10-K	001-14895	3.4	3/15/11	
10.1**	Contract Number W911QY-12-C-0117 between U.S. Department of Defense s Joint Project Manager Transformational Medical Technologies and Sarepta Therapeutics, Inc. dated August 23, 2012.					X

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10.2 Form of Stock Appreciate Right Award Agreement under the 2011 Equity Incentive Plan.

X

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Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				
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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 Vice President, Finance, Michael A. Jacobsen, 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 Vice President, Finance, Michael A. Jacobsen, 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

* In accordance with Rule 406T of Regulation S-T, the information in these exhibits is furnished and deemed not filed or a part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, is deemed not filed for purposes of Section 18 of the Exchange Act of 1934, and otherwise is not subject to liability under these sections and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such filing.

** Confidential treatment has been requested for portions of this exhibit.
Indicates management contract or compensatory plan, contract or arrangement.

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SIGNATURES

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

SAREPTA THERAPEUTICS, INC.

Date: November 7, 2012

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

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