Sarepta Therapeutics, Inc.
Form 10-K
March 03, 2014
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

x **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2013

Or

¨ **TRANSITION REPORT PURSUANT TO SECTION 13 Or 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from to

Commission file number: 001-14895

Sarepta Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**93-0797222**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

215 First Street

Suite 415

Cambridge, MA **02142**
(Address of principal executive offices) **(Zip Code)**
Registrant s telephone number, including area code: (857) 242-3700

Securities registered pursuant to Section 12(b) of the Act:

Tile of Each Class	**Name of Exchange on Which Registered**
Common Stock, $0.0001 par value	**The NASDAQ Stock Market LLC**

(The NASDAQ Global Select Market)
Securities registered pursuant to Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes x No ¨

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ¨ No x

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of the registrant s knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ¨

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

Large accelerated filer	x	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 Act). Yes ¨ No x

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2013 was approximately $1,225,412,000.

The number of outstanding shares of the registrant s common stock as of the close of business on February 24, 2014 was 37,775,169.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant has incorporated by reference into Part III of this Annual Report on Form 10-K, portions of its definitive Proxy Statement for its 2014 annual meeting to be filed with the Commission no later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

Table of Contents

Sarepta Therapeutics, Inc.

FORM 10-K INDEX

	Page
PART I	3
Item 1. Business	3
Item 1A. Risk Factors	31
Item 1B. Unresolved Staff Comments	51
Item 2. Properties	51
Item 3. Legal Proceedings	52
Item 4. Mine Safety Disclosures	52
PART II	53
Item 5. Market for Registrant s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	53
Item 6. Selected Financial Data	55
Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations	56
Item 7A. Quantitative and Qualitative Disclosures About Market Risk	68
Item 8. Financial Statements and Supplementary Data	68
Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	69
Item 9A. Controls and Procedures	69
Item 9B. Other Information	72
PART III	73
Item 10. Directors, Executive Officers and Corporate Governance	73
Item 11. Executive Compensation	73
Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	73
Item 13. Certain Relationships and Related Transactions, and Director Independence	73
Item 14. Principal Accounting Fees and Services	73
PART IV	74
Item 15. Exhibits, Financial Statement Schedules	74

Table of Contents

Forward-Looking Information

This Annual Report on Form 10-K, including the Management s Discussion and Analysis of Financial Condition and Results of Operations section in Item 7, and other materials accompanying this Annual Report on Form 10-K contain forward-looking statements or incorporate by reference forward-looking statements. The statements contained in this Annual Report on Form 10-K that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are identified by words such as believe, anticipate, expect, intend, plan, will, may, seek and other similar expressions. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other forward-looking information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our expectations regarding the development and clinical benefits of our product candidates;*
- *the results of our research and development efforts and the efficacy of our PMO-based chemistries and other RNA-based technology;*
- *our expectations regarding our ability to become a leading developer and marketer of RNA-based therapeutics;*
- *the efficacy, potency and utility of our product candidates in the treatment of rare and infectious diseases, and their potential to treat a broad number of human diseases;*
- *our expectations regarding the results of preclinical and clinical testing of our product candidates;*
- *our expectations regarding the timing for initiating a pivotal clinical study, the design of a pivotal study and for filing a new drug application (NDA) for eteplirsen with the approval of the U.S. Food and Drug Administration (FDA);*
- *our expectations regarding the timing, completion and receipt of results from our ongoing development programs;*
- *the timing of and requirements the Company must comply with to receive any required approvals from the FDA or other regulatory approvals for our products outside of the United States;*
- *the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on the Company, the development of our product candidates and the Company s financial and contractual obligations;*
- *our expectations regarding the markets for our products;*
- *acceptance of our products, if introduced, in the marketplace;*
- *the possible impact of competitive products, product development, manufacturing, commercialization and technological difficulties;*

our expectations regarding partnering opportunities and other strategic transactions;

the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs;

our plans 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;

Table of Contents

our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and statements about our future capital needs;

our ability to increase the scale of our manufacturing to provide our product to patients in larger scale clinical trials or in potential commercial quantities and meet regulatory and company quality control requirements;

our ability to operate our business without infringing the intellectual property rights of others;

our expectations about funding from government and other sources; and

other factors set forth below under the heading Risk Factors .

All forward-looking statements are based on information available to us on the date of this Annual Report on Form 10-K and we will not update any of the forward-looking statements after the date of this Annual Report on Form 10-K, except as required by law. Our actual results could differ materially from those discussed in this Annual Report on Form 10-K. The forward-looking statements contained in this Annual Report on Form 10-K, and other written and oral forward-looking statements made by us from time to time, 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 Part I, Item 1 Business and Item 1A Risk Factors of this Annual Report on Form 10-K.

Table of Contents

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including our lead product candidate, eteplirsen. We are also focused on developing therapeutics for the treatment of infectious diseases, including our lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus. By building our infectious disease programs which are primarily funded and supported by the U.S. Department of Defense (DoD), and leveraging our highly-differentiated, proprietary technology platforms, we are seeking to further develop our research and development competencies and identify additional product candidates.

Our highly-differentiated RNA-based technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe unmet medical need. Last year, we completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial. We are working with the FDA to initiate a pivotal clinical study in 2014 and to determine the possibilities under expedited regulatory programs for eteplirsen.

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

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

On July 12, 2012, our common stock began trading on The NASDAQ Global Market on a split-adjusted basis following a one-for-six reverse stock split that was effective on July 11, 2012. As of January 2, 2014, our Common Stock is quoted on The NASDAQ Global Select Market. Unless otherwise noted, all share amounts, share prices and exercise prices included throughout this report give effect to the July 2012 one-for-six reverse stock split.

Since our inception in 1980, we have incurred losses of $543.2 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on changes in warrant valuation partially offset by revenue generated from research contracts with and grants primarily from

Table of Contents

the DoD. As of December 31, 2013, we have completed all of our contracts with the DoD except for the July 2010 contract for the development of therapeutics against the Marburg virus. The period of performance for our August 2012 contract with the DoD concluded in the third quarter of 2013. In November 2012 we also entered into a consortium agreement with various parties that received an E.U. Health Innovation-1 2012 Collaborative research grant to support development of an exon 53-skipping therapeutic, based on our PMO chemistry, for which minimal revenues have been earned to date. We have not generated any material revenue from product sales to date, and there can be no assurance that revenues from product sales will be achieved. Moreover, even if we do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of December 31, 2013, we had $264.9 million of cash, cash equivalents and invested cash, comprised of $257.0 million of cash and cash equivalents and $7.9 million of restricted investments, which we believe, taking into consideration our outstanding warrants, is sufficient to fund our current operational plan for the next twelve months. Should our funding from the DoD cease or be delayed, we would likely curtail certain infectious disease research and development efforts unless additional funding was obtained. We are also likely to pursue additional cash resources through public or private financings, seeking additional government contracts, and by establishing collaborations or licensing our technologies to other companies.

We were originally incorporated in the State of Oregon on July 22, 1980 and on June 6, 2013, we reincorporated in the State of Delaware. Our principal executive offices are located at 215 First Street, Suite 415, Cambridge, MA 02142 and our telephone number is (857) 242-3700. Our common stock trades on The NASDAQ Global Select Market under the symbol SRPT.

Where You Can Find Additional Information

We make available free of charge through our corporate website, www.sarepta.com, our annual reports, quarterly reports, current reports, proxy statements and all amendments to those reports as soon as reasonably practicable after such material is electronically filed or furnished with the SEC. These reports may also be obtained without charge by submitting a written request via mail to Investor Relations, Sarepta Therapeutics, Inc., 215 First Street, Suite 415, Cambridge, MA 02142 or by e-mail to investorrelations@sarepta.com. Our Internet website and the information contained therein or incorporated therein are not intended to be incorporated into this Annual Report on Form 10-K. In addition, the public may read and copy any materials we file or furnish with the Securities and Exchange Commission, or the SEC, at the SEC s Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549 or may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. Moreover, the SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding reports that we file or furnish electronically with the SEC at www.sec.gov.

Objectives and Business Strategy

We believe that our highly-differentiated, proprietary RNA-based technology platforms can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key unmet medical needs. We intend to leverage our RNA-based technology platforms, organizational capabilities and resources to become a leading developer and marketer of RNA-based therapeutics, including for the treatment of rare and infectious diseases, with a diversified portfolio of product candidates and approved products. In pursuit of this objective, we intend to engage in the following activities:

- advancing the development of eteplirsen and our other drug candidates for the treatment of DMD to realize the product opportunities of such candidates and provide significant clinical benefits;

- successfully executing our government funded infectious disease therapeutic programs and building on and leveraging our experience with such programs to further develop our research and development capabilities and garner additional external funding; and

Table of Contents

leveraging our highly-differentiated, proprietary RNA-based technology platforms to identify product candidates in additional therapeutic areas and explore various strategic opportunities, including potential partnering, licensing or collaboration arrangements with industry partners.

Development Programs

Our currently active RNA-based drug programs are being clinically evaluated for the treatment of DMD and have also demonstrated promising antiviral activity in infectious diseases such as Marburg and H1N1 influenza in certain animal models. Our active lead product candidates are at various stages of development summarized below.

Program	Indication	Mechanism	Chemistry	Development Stage	Developer / Collaborator
Eteplirsen	DMD (exon 51)	Exon Skipping	PMO	Phase IIb*	Proprietary
AVI-7288	Marburg virus	Translation Suppression	PMO*plus*®	Phase I	Proprietary/U.S. Government
AVI-7100	H1N1 influenza virus	Translation Suppression	PMO*plus*®	Phase I	Proprietary/U.S. Government

* We announced results from our Phase IIb clinical study in eteplirsen in April 2012 and are currently conducting a long-term open label extension phase to this clinical trial.

For purposes of the table above, Development Stage indicates the most advanced stage of development that has been completed or is ongoing. In the table above, under the heading Development Stage, Phase IIb indicates clinical safety and efficacy testing in a small patient population, and Phase I indicates initial clinical safety testing in healthy volunteers or a limited patient population, or trials directed toward understanding the mechanisms or metabolism of the drug.

Duchenne Muscular Dystrophy Program

Duchenne muscular dystrophy, or DMD, is one of the most common fatal genetic disorders affecting children (primarily boys) around the world. DMD is a devastating and incurable muscle-wasting disease associated with specific mutations in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. The absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. The disease occurs in approximately one in every 3,500 male births worldwide. Females are rarely affected by the disorder. Initial symptoms, which usually appear between the ages of three and five, include progressive muscle weakness of the legs and pelvis, manifested as difficulty walking, running or climbing stairs, which eventually spreads to the arms, neck, and other areas. By age ten, braces may be required for walking, and many individuals require full-time use of a wheelchair before age 12. Eventually muscular degeneration progresses to the point of complete paralysis. Disease progression is also typically associated with respiratory muscle dysfunction and a corresponding difficulty in breathing, which may require ventilatory support, and cardiac muscle dysfunction which may lead to heart failure. DMD is ultimately fatal and death usually occurs before the age of 30. There is currently no approved disease modifying treatment or cure for DMD.

The yearly cost of care for individuals with DMD is high and increases with disease progression. Although DMD is a rare disease, we believe it represents a substantial product opportunity due to the severity and inexorable progression of the symptoms.

Our lead program is designed to address specific gene mutations that result in DMD by forcing the genetic machinery to skip over an adjacent contiguous piece (*i.e*, one or more exons) of RNA and, thus, restore the ability of the cell to express a new, truncated but functional, dystrophin protein. We believe that the expression of

Table of Contents

this truncated dystrophin protein may restore, prevent or slow deterioration of muscle function, as exemplified by the less severe muscular dystrophy phenotype, called Becker muscular dystrophy.

Eteplirsen. Eteplirsen is an antisense PMO-based therapeutic in clinical development for the treatment of individuals with DMD who have an error in the gene coding for dystrophin that can be treated by skipping exon 51. Eteplirsen targets the most frequent series of mutations that cause DMD. Eteplirsen has been granted orphan drug designation in the United States and European Union. In 2007, the FDA granted eteplirsen fast track status and we are continuing to discuss with FDA the possibility of expedited regulatory programs for eteplirsen based on the Phase IIb data. See Government Regulation for additional information.

In October 2010, we announced results from a clinical trial of eteplirsen, AVI Study 28. Data from this study were published in *The Lancet* in July 2011. AVI Study 28 was a Phase Ib/IIa open label, dose-ranging, clinical trial assessing the safety, tolerability, pharmacokinetics and exploratory efficacy of eteplirsen in ambulatory individuals with DMD. Participants in AVI Study 28 were between the ages of five and 15 with errors in the gene coding for dystrophin, which were amenable to treatment by skipping exon 51. Participants were dosed once per week for 12 weeks. A total of 19 participants were enrolled and these individuals were assigned to one of six dose cohorts of 0.5, 1.0, 2.0, 4.0, 10.0 or 20.0 mg/kg. Of the 19 participants enrolled, 18 received at least ten of the 12 doses planned in this trial. After completion of dosing, participants were followed for an additional 14 weeks. Muscle biopsies were taken before treatment and 17 participants had a second biopsy at week 14, two weeks after administration of the final dose. The primary objective of the trial was to assess the safety of eteplirsen at these doses over the 26-week duration of the trial. Secondary trial objectives included assessment of plasma pharmacokinetics, urinary elimination and exploratory endpoints evaluating biological activity and clinical performance. This trial was conducted by investigators in the United Kingdom at the University College London Institute of Child Health / Great Ormond Street Hospital in London and at the Royal Victoria Infirmary in Newcastle-Upon-Tyne. In AVI Study 28, (i) eteplirsen induced exon 51 skipping in all cohorts and new dystrophin protein expression in cohort 3; (ii) eteplirsen was well-tolerated in all participants with no drug-related serious adverse events or severe adverse events, except that one participant exhibited deteriorating cardiac function, which was considered probably disease related; (iii) adverse events were mostly mild or moderate in intensity, not dose-related, and none were considered probably or definitely related to eteplirsen; and (iv) there was no detectable immune response to newly made dystrophin.

Based on the AVI 28 study results, we initiated a Phase IIb trial for eteplirsen in August 2011, AVI 4658-us-201, or Study 201, at Nationwide Children s Hospital in Columbus, Ohio and we announced the results from this study in April 2012. This was a randomized, double-blind, placebo-controlled study to assess the efficacy, safety, tolerability and pharmacokinetics of eteplirsen administered intravenously in two different doses over 24 weeks for the treatment of ambulant boys with DMD. Exploratory clinical measures of ambulation, muscle function and strength were also captured and evaluated during the course of the trial. Study 201 included 12 participants and muscle biopsies of all participants were performed prior to initiation of treatment. The 12 participants with a genotypically-confirmed appropriate genetic mutation were randomized into one of three treatment groups with four participants in each group. The first treatment group received a weekly intravenous administration of eteplirsen at a dose of 50.0 mg/kg. The second treatment group received a weekly intravenous administration of eteplirsen at a dose of 30.0 mg/kg. The third and final treatment group received a weekly administration of placebo. Participants receiving the 50.0 mg/kg dose received a second biopsy at 12 weeks after initiation of treatment, and participants receiving the 30.0 mg/kg dose received a second biopsy at 24 weeks after initiation of treatment. The results from Study 201 determined that treatment with eteplirsen met the primary efficacy endpoint in the study. Eteplirsen administered once weekly at 30mg/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. 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 (50mg/kg once weekly). No significant improvements in clinical outcomes in the treated groups were observed compared to placebo.

Table of Contents

All participants in Study 201 were enrolled in an open-label extension study 4658-us-202, or Study 202, following the completion of Study 201 and all participants, including those from the placebo group in Study 201, are receiving either 30.0 mg/kg or 50.0 mg/kg for the duration of Study 202. The purpose of Study 202 is to evaluate the ongoing safety, efficacy and tolerability of eteplirsen. The primary efficacy endpoint was the change from baseline at week 48 in the percentage of dystrophin-positive fibers in muscle biopsy tissue as measured by immunohistochemistry. The primary clinical outcome measure was the change from baseline to week 48 on the six minute walk test, or the 6MWT. Study 202 is now in a long-term extension phase in which patients continue to be followed for safety and clinical outcomes approximately every 12 weeks through week 108 (which includes the original 28 weeks of Study 201).

On July 24, 2012, we announced interim results from Study 202 which indicated that treatment with eteplirsen over 36 weeks achieved a significant clinical benefit on the primary clinical outcome measure, the 6MWT, over a placebo/delayed treatment cohort. Eteplirsen administered once weekly at 50mg/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 50mg/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 30mg/kg weekly of eteplirsen and the placebo/delayed treatment cohort. The safety profile of eteplirsen was evaluated across all subjects through the 36 weeks eteplirsen was administered 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 Study 202 which indicated that treatment with eteplirsen met the predefined primary efficacy endpoint, increase in novel dystrophin, and achieved a significant clinical benefit on the predefined primary clinical outcome measure, the 6MWT, over the placebo/delayed treatment cohort. 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 ($p<0.009$).

In the predefined analysis of the study s intent-to-treat population on the primary clinical outcome measure, the change in 6MWT distance from baseline at week 48, 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 using mixed model repeated measures). 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.

On December 7, 2012, we announced updated data from Study 202 which showed patients treated with eteplirsen and evaluable on ambulatory measures (modified Intent to Treat population, or the mITT population) for 62 weeks maintained a statistically significant clinical benefit on the primary clinical outcome measure, the 6MWT, compared to patients who received placebo for 24 weeks followed by 38 weeks of eteplirsen treatment. In the mITT population, which includes evaluable patients from both the 30mg/kg and 50mg/kg dose cohorts,

Table of Contents

patients treated with eteplirsen for 62 weeks demonstrated a statistically significant benefit ($p \leq 0.007$) of 62 meters over the placebo/delayed-treatment cohort using a mixed-model repeated measure statistical test. The mITT population utilized for the 62 week analysis consisted of 10 of the enrolled 12 patients (4 eteplirsen-treated patients receiving 50 mg/kg weekly, 2 eteplirsen-treated patients receiving 30 mg/kg weekly, and 4 placebo/delayed-treatment patients), and excluded two patients who showed signs of rapid disease progression and lost ambulation by week 24. The eteplirsen treatment cohort (n=6) continued to show disease stabilization with less than a 5% decline in walking distance on the 6MWT from baseline. The placebo/delayed-treatment cohort (n=4) also demonstrated stability in walking distance from week 36 through week 62 with a less than 10 meter change over this timeframe, the period in which dystrophin was likely produced, with confirmation of significant dystrophin levels at week 48 through analysis of muscle biopsies in these patients.

The safety profile of eteplirsen was evaluated across all patients through week 62 and there were no clinically significant treatment-related adverse events, no serious adverse events, and no discontinuations. One patient had a laboratory treatment-related adverse event, a transient elevation of urine protein on a urine dipstick test, however this elevation was not observed on a 24-hour urine protein measurement and resulted in no clinical symptoms or interruption of treatment. This patient did not show elevations of the specific renal markers of cystatin C or KIM-1. Across both the treatment and placebo/delayed treatment cohorts there is evidence of continued stabilization on pulmonary function tests, echocardiogram, muscle strength and clinical laboratory tests over the 62 weeks.

Results from the mITT population, which combines the evaluable eteplirsen-treated patients across the 30mg/kg and 50mg/kg cohorts, have been previously reported and will be used as the primary assessment of ambulatory clinical measures for the remainder of Study 202. Given there was no significant difference between the 30 mg/kg and 50 mg/kg arms on the production of dystrophin through 48 weeks, we believe this mITT population is the most appropriate to assess dystrophin production and its potential predictive benefits on ambulatory clinical outcomes, such as the 6MWT.

On April 5, 2013, we announced that, after 74 weeks, patients in the 30 mg/kg and 50 mg/kg dose cohorts in the mITT population (n=6) showed a statistically significant treatment benefit of 65.2 meters ($p \leq 0.004$) when compared to the placebo/delayed-treatment cohort (n=4). The eteplirsen-treated patients in the mITT population demonstrated less than a 5 percent decline (13.4 meters) from baseline in walking ability. After experiencing a substantial decline earlier in the study, the placebo/delayed-treatment cohort also demonstrated stabilization in walking ability from week 36 through 74, the period in which meaningful levels of dystrophin were likely produced, with a less than 10 meter decline over this timeframe. Through 74 weeks, eteplirsen was well tolerated and there were no clinically significant treatment-related adverse events, serious adverse events, hospitalizations or discontinuations. As previously reported at 62 weeks, one patient had a transient elevation of urine protein on a laboratory urine dipstick test, which resolved and resulted in no clinical symptoms. The patient continued treatment without interruption and remained free of proteinuria through week 74. Across both the eteplirsen (mITT) and placebo/delayed-treatment cohorts, there was evidence of continued stabilization on clinical laboratory tests, echocardiogram, pulmonary function tests and muscle strength through 74 weeks of participating in Study 202.

On June 19, 2013, we announced that after 84 weeks, patients in the 30 mg/kg and 50 mg/kg dose cohorts in the mITT population (n=6) showed a statistically significant treatment benefit of 46.4 meters ($p \leq 0.045$) when compared to the placebo/delayed-treatment cohort (n=4). The eteplirsen-treated patients in the mITT population demonstrated less than a 6 percent decline (20.5 meters) from baseline in walking ability. The placebo/delayed-treatment cohort also demonstrated stabilization in walking ability from Week 36 through 84, the period from which meaningful levels of dystrophin were likely produced, with an increase of 3.3 meters over this timeframe. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days. Through 84 weeks, eteplirsen was well tolerated and there were no clinically significant treatment-related adverse events, no serious adverse events, hospitalizations or discontinuations. One boy in the placebo/delayed-treatment cohort was not able to perform the 6MWT at the Week 84 clinic visit due to a physical injury unrelated

Table of Contents

to treatment, and therefore had no 6MWT data captured at the Week 84 time point. The boy has recovered from the injury, continues to be ambulatory and is expected to be evaluated on the 6MWT at future clinic visits. Across all patients in the eteplirsen and placebo/delayed-treatment cohorts, there was evidence of continued stabilization on clinical laboratory tests, echocardiograms, pulmonary function tests and measures of muscle strength through 84 weeks of participating in Study 202.

On September 26, 2013, we announced that after 96 weeks, patients in the 30 mg/kg and 50 mg/kg eteplirsen cohorts in the mITT population (n=6) experienced less than a 5 percent decline (17.5 meters) from baseline in walking ability. A statistically significant treatment benefit of 70.8 meters ($p \leq 0.001$) was observed for the mITT population compared with the placebo/delayed-treatment cohort (n=4). The placebo/delayed-treatment cohort also demonstrated stabilization in walking ability from Week 36 through 96, the period from which meaningful levels of dystrophin were likely produced, with a decline of 18.5 meters over this timeframe. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days. As previously reported, a boy in the placebo/delayed-treatment cohort was not able to perform the 6MWT at the Week 84 clinic visit due to a broken ankle assessed by the investigator as a treatment-unrelated adverse event. Although this boy received rehabilitation and was able to perform the 6MWT, his walking ability at the time of the test had not returned to the level observed prior to the injury, and this lower 6MWT distance contributed to the overall decline in the placebo/delayed-treatment cohort. The decline in walking distance observed in this cohort from Week 36 improves from a decline of 18.5 meters to a decline of 4.7 meters when this patient s 96-week test score is excluded from the analysis. Through 96 weeks, eteplirsen was well tolerated and there were no reported clinically significant treatment-related adverse events, no treatment-related serious adverse events, hospitalizations or discontinuations. Across patients in the eteplirsen and placebo/delayed-treatment cohorts, there is evidence of continued stabilization on clinical laboratory tests, echocardiograms, pulmonary function tests and measures of muscle strength through 84 weeks of participating in Study 202.

On January 15, 2014, we announced that at 120 weeks, patients in the 30 mg/kg and 50 mg/kg eteplirsen cohorts who were able to perform the 6MWT (modified Intent-to-Treat or mITT population; n=6) experienced a decline of 13.9 meters, or less than 5 percent, from baseline in walking ability. A statistically significant treatment benefit of 64.9 meters ($p \leq 0.006$) was observed for the mITT population compared with the placebo/delayed-treatment cohort (n=4). The placebo/delayed-treatment cohort also demonstrated stabilization in walking ability for more than 1.5 years, from Week 36 through 120, the period from which meaningful levels of dystrophin were likely produced, with a decline of 9.5 meters over this timeframe. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days. In addition, on February 5, 2014, we announced that results through more than two years of treatment showed stable pulmonary function in the Intent-to-Treat (ITT) study population (N=12). Through 120 weeks, eteplirsen was well tolerated and there were no reported clinically significant treatment-related adverse events and no treatment-related serious adverse events. In addition, there were no treatment-related hospitalizations or discontinuations.

We will continue to have discussions with the FDA during the first quarter of 2014 regarding the design of the pivotal study, the clinical results from our Phase IIb study of eteplirsen and the possibility of expedited regulatory programs for eteplirsen based on the Phase IIb data. Based on feedback from these meetings, we will make a determination regarding the most appropriate regulatory path for pursuing regulatory approval of eteplirsen. Any such determination will be further informed by subsequent meetings with the FDA. Regardless of the approval process and path ultimately pursued, we anticipate initiating a pivotal clinical study for eteplirsen and commencing dosing during the second or third quarter of 2014.

Pan-Exon Strategy. In addition to our lead product candidate, eteplirsen, we are pursuing development of additional exon-skipping drugs, to support our broad-based development program for the treatment of DMD. For example, as of December 31, 2013, we have pre-clinical studies under way for exon 45-skipping and exon 53-skipping therapeutics, a lead sequence identified for an exon-50 skipping therapeutic and lead sequence selection under way for exon 44, exon 52, exon 55 and exon 8-skipping therapeutics.

Table of Contents

To support certain activities to enable an Investigational New Drug, or IND, for an exon 45-skipping therapeutic, we are collaborating with Children s National Medical Center in Washington, D.C. and the Carolinas Medical Center (in Charlotte, N.C.). This collaboration is funded primarily through two grants, one from DoD s Congressionally Directed Medical Research Program to Children s National Medical Center and the other from the National Institute of Neurological Disorders and Stroke to the Carolinas Medical Center. This funding is intended to pursue the most promising treatments for DMD. The collaboration will support a series of Good Laboratory Practice, or GLP, toxicology studies for an exon 45-skipping drug candidate based on our PMO chemistry.

To support certain clinical proof of concept studies and IND-enabling activities for an exon 53-skipping therapeutic, we announced in November 2012 that we are collaborating with University College London s scientist, Professor Francesco Muntoni, M.D., the Dubowitz Neuromuscular Centre, the Institute of Child Health and other scientists from the European Union and the United States. In connection with this collaboration, the consortium received an E.U. Health Innovation-1 2012 Collaborative research grant to support development of an exon 53-skipping therapeutic, based on our PMO chemistry. Targeting exon 53 with this technology will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping (deletion of exons 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, or 52) by potentially restoring the cellular machinery s ability to produce a functional dystrophin protein.

To support certain IND-enabling activities for an exon 50-skipping therapeutic, we entered into a Cooperative Research and Development Agreement, or CRADA, in August 2012 with the National Institutes of Health, or NIH, which was anticipated to be supported through in-kind research conducted either by the Therapeutics for Rare and Neglected Diseases program or by contract research organizations. We and NIH mutually agreed to terminate the CRADA in February 2013 and we are now developing exon 50 utilizing our own research and development capabilities. We do not anticipate any significant changes in IND filing timelines due to the termination.

These collaborations and our DMD program, which includes eteplirsen, are part of our larger pan-exon strategy for the development of drug candidates to address the most prevalent exon deletions in the DMD population. Because the majority of DMD patients have exon deletions that cluster together, a small number of exon-skipping therapies will potentially be disease-modifying for a relatively large percentage of DMD patients. Approximately 75-80% of the total DMD population is potentially treatable with exon-skipping therapeutics. According to an article by Aartsma-Rus et. al published in 2009 in the Human Genome Variation Society Journal, of the exon skipping amenable population, exon 51 skipping is applicable to the largest sub-group, equal to approximately 13%. Skipping of exons 50, 45, 44, 52, 55 and 8 is applicable to approximately 4%, 8%, 6%, 4%, 2% and 2%, respectively.

Infectious Disease Programs

With the financial support of the U.S. government, we are currently implementing our RNA-based technology platforms in our infectious disease programs for the development of therapeutics to treat infectious diseases, such as Marburg and influenza. In the past, DoD has provided significant financial support for our development of therapeutics designed to treat Ebola, Marburg and influenza viruses. We have also entered into an agreement with the National Institute of Allergy and Infectious Diseases, or NIAID, part of NIH, under which NIAID is providing clinical support for the development of our therapeutic candidate for the treatment of influenza.

Our current arrangement with DoD supports development of our Marburg drug candidate, AVI-7288, including activities necessary to obtain approval of an NDA by the FDA, if DoD exercises all of its options under the arrangement. 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 and completed the performance of our obligations under this agreement in the third quarter of 2013. Under a separate arrangement,

Table of Contents

DoD similarly provided funding to advance the development of our H1N1 influenza drug candidate, AVI-7100, through an IND application with the FDA and to preclinically evaluate its therapeutic potential against H5N1 (avian flu), Tamiflu® resistant H1N1 (pandemic flu) and H3N2 (seasonal flu) which concluded in 2011. In December 2012, we entered into an agreement with NIAID to support further development of AVI-7100. Under the agreement, NIAID researchers are allowed to proceed with a Phase I, study to assess the safety, tolerability and pharmacokinetics of single and multiple doses of AVI-7100 in healthy volunteers. Per the terms of the agreement, we provided AVI-7100 to NIAID and in return, we will have the right to use the data from this clinical study to support future development of AVI-7100.

Without continued government support of these programs we would likely significantly curtail our development efforts with respect to these programs. Future funding and support is subject to availability of budgeted funds from DoD and the Department of Health and Human Services, or DHHS, as government support for some of our infectious disease programs has previously been discontinued or not renewed due to government budget constraints. For example, our current arrangement with DoD initially provided for support of the development of our Ebola virus drug candidate; however, on October 2, 2012, the Company received notice from DoD that the Ebola portion of the arrangement 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 arrangement with DoD 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 arrangement with DoD and the Marburg portion remains actively in development under the DoD arrangement. Additionally, the period of performance for our June 2010 H1N1 influenza contract with DoD expired in June 2011. Additional research for this antiviral program is being conducted by NIAID as described elsewhere in this report.

In the periods presented in this report, substantially all of our revenues were derived from research and development contracts with and grants from the U.S. government. As of December 31, 2013, we had completed all of our contracts with the U.S. government except for the Marburg portion of the July 2010 agreement for the development of therapeutics against Marburg and Ebola viruses. For a more detailed description of our contracts with the U.S. government, see Management s Discussion and Analysis of Financial Condition and Results of Operations U.S. Government Contracts and Note 6 Government Contracts of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Hemorrhagic Fever Virus Programs. Our infectious disease therapeutic programs use our translation suppression technology and apply our proprietary PMO-*plus*® chemistry backbone, an advanced generation of our base PMO chemistry backbone that selectively introduces positive backbone charges to improve selective interaction between the drug and its target. Our translation suppressing technology is based on Translation Suppressing Oligomers which are PMO-based compounds that stop or suppress the translation of a specific protein by binding to their specific target sequence in mRNA. We are pursuing development and regulatory approval of our Marburg hemorrhagic fever virus product candidate under the FDA s Animal Rule. The Animal Rule provides that under certain circumstances, where it is unethical or not feasible to conduct human efficacy studies, the FDA may grant marketing approval based on adequate and well-controlled animal studies when the results of those studies establish that the drug or biological product is reasonably likely to produce clinical benefit in humans. Demonstration of the product s safety in humans is still required. See Government Regulation Animal Rule for additional information.

Marburg virus. AVI-7288 is designed for post-exposure prophylaxis after documented or suspected exposure to Marburg virus. Marburg hemorrhagic fever is a severe and often fatal disease in humans that was first recognized in 1967. It is caused by an RNA virus of the Filoviridae family and is understood to be endemic to Africa. The Marburg virus is classified as a Category A bioterrorism agent by the Centers for Disease Control and Prevention, or CDC, and was determined to be a material threat to national security by the Secretary of Homeland Security in 2006. Onset of the disease is often sudden and the symptoms include fever, chills, nausea, vomiting, chest pain and diarrhea. Increasingly severe symptoms may also include massive hemorrhaging and multiple organ dysfunction. There are currently no treatments for Marburg virus infection beyond supportive care

Table of Contents

and the mortality rate is very high. For Marburg virus infection, our lead product candidate is currently AVI-7288. Previously, our lead product candidate for Marburg virus infection was AVI-6003 which is a combination of AVI-7287 and AVI-7288; however, in February 2012, we announced that we received agreement from the FDA to remove AVI-7287 and we are now proceeding with a single oligomer approach, AVI-7288, given that efficacy in non-human primates has been demonstrated to be attributable to this single oligomer. During the 2012 fiscal year, we completed Phase I single ascending-dose studies in healthy volunteers with our candidates for the treatment of Ebola virus and Marburg virus and in July 2012, we announced results from a non-human primate study of the efficacy of AVI-7288. In September 2012, we announced that the FDA has granted fast track status for the development of AVI-7288 and our product candidate against Ebola, AVI-7537. In March 2013, with the support of DoD s Joint Project Manager Medical Countermeasure Systems, in non-human primate study, we completed an evaluation of the feasibility of in intramuscular route of administration using AVI-7288, including an evaluation of the tolerability, pharmacokinetics, and efficacy of intramuscular AVI-7288. The data showed that intramuscular administration of AVI-7288 resulted in survival rates up to 100 percent in treated subjects, similar to efficacy observed in previous studies that evaluated the drug when administered by intravenous injection. In May 2013, we initiated dosing of AVI-7288 in a Phase I multiple ascending dose study which we expect to complete in the first quarter of 2014. In February 2014, we announced positive safety results from a Phase I multiple ascending dose study of AVI-7288 in healthy volunteers.

Ebola virus. AVI-7537 is a single agent designed for post-exposure prophylaxis after documented or suspected exposure to the Ebola virus. The hemorrhagic fever caused by the Ebola virus is severe and often fatal in humans and there are currently no treatments for Ebola beyond supportive care. AVI-6002, a combination of AVI-7537 and AVI-7539, was previously our product candidate for the Ebola virus. However, based on our evaluation of the efficacy of AVI-7537 as a single agent versus a combination with AVI-7539 which demonstrated that efficacy could be attributed to the single oligomer AVI-7537, we transitioned our focus to this product candidate in 2012. Although we believe AVI-7537 has the potential to be a therapeutic option for the Ebola virus, we suspended our development efforts with respect to our Ebola program after the August 2012 stop-work order and subsequent termination for convenience by DoD of support for this program in 2012. The termination only applies to the Ebola portion of our arrangement with DoD and the Marburg portion remains in effect.

Development Status of Hemorrhagic Fever Virus Programs. Non-human primates infected with Marburg virus and treated with our precursor product candidate, AVI-6003, achieved 100% survival and primates infected with Ebola virus and treated with, AVI-6002, achieved 80% survival, in each case compared to universal lethality in both control groups. In addition to survival, primates treated with AVI-6002 and AVI-6003 have demonstrated decreases in levels of viremia, in harmful inflammatory indicators and in virus induced liver damage. Additional data have also demonstrated that the surviving animals were resistant to viral infection after subsequent injection with the virus.

During the 2012 fiscal year, we completed Phase I single ascending-dose studies in healthy adult volunteers with its drug candidates for the treatment of Ebola virus and Marburg virus demonstrating positive safety data for each therapeutic candidate. In February 2012, we announced positive safety results from all six cohorts of our Phase I single ascending dose trials of AVI-6002 and AVI-6003. For each group, safety, clinical laboratory and renal biomarker results through five days after treatment were reviewed by an independent Data and Safety Monitoring Board, or DSMB, which issued recommendations for both studies to progress as planned to multiple ascending dose studies after no safety concerns were identified. The Phase I single ascending dose trials were designed to characterize the safety, tolerability and pharmacokinetics of each therapeutic candidate in healthy adult volunteers. In the two studies, a total of 60 healthy human subjects (five per group) were enrolled into six sequential dose groups (0.01, 0.1, 1.0, 3.0, 6.0 or 9.0 mg/kg). Within each group, four subjects received the indicated dose of the therapeutic and one subject received placebo. Final, unblinded safety and pharmacokinetic results for all subjects were completed in 2012.

In July 2012, we announced that AVI-7288 demonstrated up to 100% survival in a non-human primate study exploring the drug s effect when the initiation of treatment is delayed to various time points post-infection.

Table of Contents

This study showed a high degree of survival between 83% and 100% in each of four post-exposure cohorts that received daily treatments with AVI-7288 beginning one-, 24-, 48-, or 96-hours after infection, compared to 0% survival in the placebo-treated control group.

In March 2013, we announced positive results from a non-human primate study of AVI-7288. The data showed that intramuscular administration of AVI-7288 resulted in survival rates up to 100 percent in treated subjects, similar to efficacy observed in previous studies that evaluated the drug when administered by intravenous injection.

We initiated a Phase I multiple ascending dose study in May 2013, designed to characterize the safety, tolerability and pharmacokinetics of multiple doses of AVI-7288 in healthy adult volunteers. The randomized, double-blind placebo-controlled study has been overseen by an independent DSMB, which reviewed the safety and clinical laboratory data after each dose cohort prior to enrolling the next higher dose cohort. The final cohort completed dosing in the first quarter of 2014. In February 2014, we announced positive safety results from a Phase I multiple ascending dose study of AVI-7288 in healthy volunteers. An independent DSMB reviewed the safety profile and recommended proceeding with further development of AVI-7288 at doses up to 16 mg/kg. Subject to approval under the existing contract with the Joint Project Manager Transformational Medical Technologies program (renamed Medical Countermeasure Systems in 2013) of the DoD (the JMP-MCS), further development of AVI-7288 is planned pursuant to FDA s Animal Efficacy Rule.

Influenza Program. Our infectious disease therapeutic programs are also focused on the development of our product candidates designed to treat pandemic influenza viruses. AVI-7100 is our lead product candidate for the treatment of influenza and employs our PMO-*plus*® technology. In December 2012, we entered into an agreement with NIAID which permits NIAID to conduct a Phase I single and multiple ascending dose study with AVI-7100. In June 2010, we were awarded a contract under DoD s Transformational Medical Technologies, or TMT, program (renamed Medical Technologies Systems in 2013), which funded our activities to develop AVI-7100 as a medical countermeasure against the pandemic H1N1 influenza virus. The period of performance for this contract ended in June 2011. See Management s Discussion and Analysis of Financial Condition and Results of Operations U.S. Government Contracts and Note 6 Government Contracts of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for additional information.

Symptoms of H1N1 influenza include fever, cough, runny nose, headache, chills and fatigue. Many people infected with H1N1 also have respiratory symptoms without a fever. Severe illness and deaths have also occurred. The CDC estimated that between April 2009 and April 2010 there were up to 89 million cases of H1N1 infection in the United States. The CDC also estimated that there were up to 403,000 H1N1-related hospitalizations in the United States during the same time period.

The TMT program established a contract with us to conduct a rapid response exercise against a real-world emerging threat like the pandemic H1N1 virus. The intent of the exercise was to demonstrate our capability to efficiently respond to a real-world emerging viral threat by rapidly designing and producing multiple therapeutic candidates and evaluating preclinical efficacy. Initially the exercise involved identifying target sequences against H1N1, designing several drug candidates utilizing proprietary derivatives of our PMO chemistry, and then manufacturing the candidates in sufficient quantity for limited preclinical testing. We successfully accomplished these steps in approximately one week, demonstrating our ability to rapidly respond to a real-world viral threat utilizing our RNA-based technology platforms.

Subsequently, we evaluated the preclinical activity of AVI-7100 and found that it showed a favorable safety profile in ferrets, rats and monkeys. In separate ferret studies, AVI-7100 demonstrated activity as a potentiator of Tamiflu® and activity towards preventing transmission of Tamiflu®-resistant H1N1.

In June 2011, we initiated dosing of AVI-7100 via intravenous infusion in single-ascending doses in up to 48 healthy adult volunteers. The first dose cohort in this Phase I, randomized, double-blind, placebo-controlled

Table of Contents

study was completed and received a favorable review from the DSMB to proceed to the next dose escalation. The period of performance under this DoD contract subsequently ended and, as a result, continued development was suspended until we entered into the clinical trial agreement with NIAID.

Under the December 2012 agreement with NIAID, NIAID researchers are allowed to proceed with a Phase I, double-blind, placebo-controlled, dose-escalating study to assess the safety, tolerability and pharmacokinetics of single and multiple doses of an intravenous formulation of AVI-7100 in healthy volunteers. Per the terms of the agreement, we provided AVI-7100 to NIAID and in return, we will have the right to use the data from this clinical study to support future development of AVI-7100.

Discovery Stage Program Overview

Our PMO-chemistries are highly-differentiated from other RNA technologies, including antisense, siRNA and RNAi. Unlike these technologies, which are often used for down-regulation of gene expression, ours can be used to selectively up-regulate or down-regulate the expression of proteins involved in human diseases and disorders, or direct the production of novel proteins with clinically relevant properties.

In addition to our pan-exon strategy for DMD, our preclinical research efforts are focused on the creation of product candidates for the treatment of other neuromuscular, infectious and rare diseases.

Chemistry Technology

Our core chemistry is based on phosphorodiamidate-linked morpholino oligomers, or PMOs, and this core chemistry has been safely dosed in over 400 patients. PMOs are synthetic molecules based on a fundamental redesign of the natural nucleic acid structure of DNA and RNA. PMOs bind to complementary sequences of RNA by standard Watson-Crick nucleic acid base-pairing and control gene expression by steric blockade of targeted RNA. Structurally, the key difference between PMOs and naturally occurring DNA and RNA is that while PMOs, like DNA and RNA, have nucleic acid bases, those bases are bound to synthetic morpholine rings instead of deoxyribose (in DNA) or ribose (in RNA) rings, and they are linked through phosphorodiamidate groups instead of phosphate groups. Replacement of anionic phosphates with the charge-neutral phosphorodiamidate groups eliminates ionization in the usual physiological pH range, thus PMOs in organisms or cells are uncharged molecules. Because of these modifications, PMOs are especially resistant to degradation by plasma and intracellular enzymes. Unlike some other RNA-based technologies, including siRNAs and other types of antisense, PMOs rely on steric blocking rather than cellular enzymatic activity for their biological effects. In this way, PMOs operate fundamentally differently from other well-known RNA-based technologies.

We have developed three new PMO-based chemistry platforms in addition to our original PMO-based technology. We believe that the novel, favorable characteristics intrinsic in these new platforms will allow for the development of drug candidates with superior delivery, specificity, therapeutic windows and drug-like properties.

PPMO. The first of these novel chemistries is based on peptide conjugated PMOs, or PPMOs, in which cellular uptake of the PMO component, as well as its potency and specificity of tissue targeting, may be significantly enhanced.

PMO-plus®. The second of these chemistries, PMO*plus*®, includes the addition of selectively introduced positive charges to the PMO backbone. We believe that while PMO-*plus*® has potentially broad therapeutic applications, it has thus far shown to be particularly effective in increasing the potency of PMO-based oligomers.

PMO-X . The third of these chemistries, PMO-X , involves novel, selective, and proprietary backbone chemistry modifications. We believe PMO-X may provide enhanced in vivo potency for our drug candidates, as well as greater flexibility in modulation of their tissue targeting, cellular delivery and uptake.

Table of Contents

We intend to continue to support our internal research and development efforts in order to advance our proprietary chemistries and to develop new analogues that may provide additional benefits in key characteristics of drug performance.

Mechanisms of Action

Humans have far fewer genes than the number of unique proteins expressed in the human proteome. The genetic information stored in human DNA is not contiguous. Short DNA stretches, called exons that code for fragments of the protein are separated by long non-coding pieces of DNA called introns. During processing of precursor or pre-mRNA, which is copied from the DNA template, introns are removed and exons spliced together to create the mature mRNA, from which a functional protein can be made. Pre-mRNA copied from a gene can be spliced through alternative paths, such that different exons are combined, creating multiple mRNAs and, hence, generate multiple proteins from a single gene.

Our PMO-based molecules are designed to sterically block the access of cellular machinery to pre-mRNA and mRNA without degrading the RNA. Through this selective targeting, two distinct biologic mechanisms of action can be initiated: (1) modulation of pre-mRNA splicing (also commonly described as splice switching, exon skipping or directed alternative splicing) and (2) inhibition of mRNA translation (also commonly described as translation suppression). Through these mechanisms, steric-blocking oligonucleotides can repair defective RNA, up or down-regulate the production of selected proteins, or produce novel or remodeled proteins.

Material Agreements

We believe that our RNA-based technology could be broadly applicable for the potential development of pharmaceutical products in many therapeutic areas. To further exploit our core technology, we have and may continue to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations and pharmaceutical and biotechnology companies for specific molecular targets or selected disease indications. We may also selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements.

U.S. Department of Defense and DHHS Agreements

We currently have contracts with DoD and its agencies and DHHS and its agencies that fund and/or support our programs. For a more detailed description of our contracts with the U.S. government, see Management s Discussion and Analysis of Financial Condition and Results of Operations U.S. Government Contracts below and Note 6 Government Contracts of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K. Our contracts with the government may be subject to renegotiation or termination at the election of the government. For a description of the risks we face relating to such rights of the government see Risk Factors Risks Relating to Our Business .

University of Western Australia

In November 2008, we entered into an exclusive license agreement with the University of Western Australia, or UWA, for certain patents and technical information relating to the use of certain antisense sequences for the treatment of DMD and in April 2013, we entered into an agreement with UWA under which this license agreement was amended and restated, referred to in this report as the Amended and Restated UWA License Agreement. The Amended and Restated UWA License Agreement grants us specific rights to the treatment of DMD by inducing the skipping of certain exons. Our lead clinical candidate, eteplirsen, falls under the scope of the license granted under the Amended and Restated UWA License Agreement. Any future drug candidates developed for the treatment of DMD by exon skipping may or may not fall under the scope of the Amended and Restated UWA License Agreement.

Table of Contents

Under the Amended and Restated UWA License Agreement, we are required to meet certain performance diligence obligations related to development and commercialization of products developed under the license. We believe we are currently in compliance with these obligations. In 2013, we made an initial upfront payment to UWA of $1.1 million upon execution of the Amended and Restated UWA License Agreement. We may be required to make additional payments to UWA of up to $6 million in the aggregate based on successful achievement of certain regulatory and commercialization-related milestones of eteplirsen and up to five additional product candidates and also may be required to pay royalties ranging from a fraction of a percent to the low single-digit percentages on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. We are not under any current obligation to make royalty payments to UWA until a product candidate is approved for commercial sale.

The terms of the Amended and Restated UWA License Agreement will expire on a country-by-country basis on the expiration date of the last to expire valid claim or patent within the patents licensed to us under this agreement or upon the earliest to occur of the following:

- failure by us or UWA to cure a breach or default of any material obligation we each have under the agreement after notice from the non-breaching party within the specified time periods;
- a mutual agreement to terminate the agreement;
- by UWA in the event a party passes a resolution to wind-up or if a receiver, administrator, trustee or person performing similar functions is appointed by a court or liquidator over any of our assets; or
- upon our notice to UWA that we no longer desire to commercialize products covered under the agreement.

Currently, the latest date on which an issued patent covered by our agreement with UWA expires is April 2026, however, pending patents could result in a later expiration date.

Strategic Alliances

Isis Ercole Agreement

In May 2003, Ercole Biotechnology, Inc., or Ercole, and Isis Pharmaceuticals, Inc., or Isis, entered into a collaboration and license agreement related to RNA splicing. Research collaboration activity defined in the agreement expired in 2006. In March 2008, we acquired all of the stock of Ercole in exchange for 5,811,721 shares of our common stock, which was valued at approximately $8.4 million, and the assumption of approximately $1.8 million in liabilities of Ercole. We also issued warrants to purchase our common stock (also classified as equity), which were valued at $437,000, in exchange for certain outstanding warrants issued by Ercole. In connection with the March 2008 acquisition, we assumed Ercole s obligations under the Isis agreement. This agreement contains several cross-licenses between the parties granting each party certain exclusive and nonexclusive rights under a selected set of the other parties patents and patent applications for the research, development, and commercialization of antisense therapeutics using RNA splicing with respect to certain gene targets.

Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, we may be obligated to make milestone payments to Isis of up to $23.4 million in the aggregate for each product developed under a licensed patent under this agreement.

As of December 31, 2013, we have not made, and are not under any current obligation to make, any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. The range of percentage royalty payments required to be made by us under the terms of this agreement is from a fraction of a percent to mid single-digit percentages. We believe that our DMD, Ebola, Marburg and influenza programs will not fall under the scope of this agreement and therefore will not be subject to milestone or royalty obligations under its provisions.

Table of Contents

Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, Isis may be obligated to make milestone payments to us of up to $21.1 million in the aggregate for each product developed under a licensed patent under this agreement. As of December 31, 2013, Isis has not made, and is not under any current obligation to make, any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. The percentage royalty payments required to be made by Isis under the terms of this agreement is a fraction of a percent. As to any product commercialized under the agreement, the agreement will terminate on the expiration date of the last to expire licensed patent covering such product. The last to expire Sarepta owned patent covered under this agreement expires on September 9, 2014. The last Isis owned patent covered under this agreement expires on March 27, 2028. In addition, either party may terminate this agreement in the event:

- a material breach by the other party is not cured within a specified period of time; or
- the other party commences bankruptcy, reorganization, liquidation or receivership proceedings or upon the assignment of a substantial portion of the assets for the benefit of creditors by the other party with certain exceptions.

Charley s Fund Agreement

In October 2007, Charley s Fund, Inc., or Charley s Fund, a nonprofit organization that funds drug development and discovery initiatives specific to DMD, awarded us a $2.45 million research grant and, in May 2009, the grant authorization was increased to a total of $5.0 million. Pursuant to the related sponsored research agreement, the grant was provided to support the development of product candidates related to exon 50 skipping using our proprietary exon skipping technologies. As of December 31, 2013, Charley s Fund has made payments of approximately $3.4 million to us. Revenue associated with this research and development arrangement is recognized based on the proportional performance method, using the payment received method. To date, we have recognized $60,000 as revenue, but did not recognize any revenue for the years ended December 31, 2013, 2012 and 2011. We do not expect to receive any incremental funding under the grant and have deferred $3.3 million of previous receipts which are anticipated to be recognized as revenue once we complete the remaining milestones.

Under the terms of the sponsored research agreement, as amended, if we and any of our strategic partners elect to discontinue the development and commercialization of any product containing any molecular candidate arising or derived from the research sponsored by Charley s Fund for reasons other than safety or efficacy, we must grant to Charley s Fund an exclusive, royalty-bearing, fully-paid, worldwide license, with right of sublicense, to any such product. Depending on whether and when Charley s Fund obtains a license to any such product, percentage royalty payments on net sales required to be made by Charley s Fund to us under the terms of the sponsored research agreement, as amended, would be in the mid-single-digits. Under the terms of the sponsored research agreement, as amended, if we are able to successfully commercialize any molecular candidate arising or derived from the research sponsored by Charley s Fund either through sales of products or through licensing or partnership arrangements with a third party that include rights for such third party to sell, distribute, promote or market such products or the underlying intellectual property, then we are obligated to repay the research funds paid to us by Charley s Fund, up to an amount equal to the total amount of funds provided by Charley s Fund to us. In connection with this repayment obligation, we agreed that we would pay a mid range single-digit percentage royalty on net sales of products containing any molecular candidate arising or derived from the research sponsored by Charley s Fund and a mid-teens amount of any upfront cash and/or milestone payments received from a licensing or partnership arrangement with a third party with respect to such products (in each case, up to an amount equal to the total amount of funds provided by Charley s Fund to us). This agreement will terminate by its own terms at the completion of the research being sponsored by Charley s Fund. The Sarepta technology upon which the agreement is based is covered by certain patents, the last of which expires following the termination of the agreement.

Previously, we noted unexpected toxicology findings in the kidney as part of our series of preclinical studies for AVI-5038, our PMO-based candidate designed for the treatment of individuals with DMD who have an error

Table of Contents

in the gene coding for dystrophin that can be treated by skipping exon 50. We have conducted additional preclinical studies and have not alleviated the toxicity problem. Pursuant to the terms of our agreement with Charley s Fund, the receipt of additional funds is tied to the satisfaction of certain clinical milestones. Because of the toxicity issues with AVI-5038, satisfaction of the additional milestones under the agreement is unlikely and we do not expect to receive any additional funds from Charley s Fund.

Manufacturing

We believe we have developed proprietary manufacturing techniques that allow synthesis and purification of our product candidates to support clinical development. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. We do not have, and do not intend to establish in the near term, any of our own internal mid-to-large scale manufacturing capabilities to support our product candidates.

For our current development programs we have entered into supply agreements with certain large pharmaceutical manufacturing firms for the production of the custom raw materials required for PMO production and the active pharmaceutical ingredients, or APIs, for our product candidates.

For our DMD program, we are working with our existing manufacturers to increase our API production capacity from small-scale to mid-scale. During 2014, we will also evaluate whether to increase our API production capacity to a commercial scale. This decision will depend in significant part on our discussions with the FDA in 2014 as well as our expectations regarding clinical trial needs and the potential feasibility and timing of an NDA filing and subsequent commercialization.

There are a limited number of companies that can produce raw materials and APIs in the quantities and with the quality and purity that we require for our DMD development efforts. Due to their technical expertise, experience in manufacturing our product candidates and sophistication of their manufacturing facilities and quality systems, we are considering our existing manufacturers, as well as other manufacturers with relevant expertise, for the further scale-up of the production of raw materials and APIs for our DMD program. Establishing a relationship with alternative suppliers can be a lengthy process and might cause delays in our development efforts. If we are required to seek alternative supply arrangements, the resulting delays and potential inability to find a suitable replacement could materially and adversely impact our business.

Manufacturers and suppliers of product candidates are subject to the FDA s current Good Manufacturing Practices, or cGMP, requirements, and other rules and regulations prescribed by foreign regulatory authorities. We depend on our third-party suppliers and manufacturers for continued compliance with cGMP requirements and applicable foreign standards.

Sales and Marketing Strategy

We have not obtained regulatory approval for any of our product candidates and thus have not yet established a commercial organization or distribution capabilities. Due to the rare nature of DMD and the lack of disease-modifying treatments, patients suffering from DMD, together with their physicians, often have a high degree of organization and are well informed, which may simplify the identification of a target population for eteplirsen, our lead product candidate, if it is approved. We believe that, if approved for commercial sale, it will be possible to commercialize eteplirsen with a relatively small specialty sales force that calls on the physicians, foundations and other patient-advocacy groups focused on DMD. Our current expectation is to commercialize eteplirsen ourselves in the United States and plan to recruit a sales force and take other steps to establish the necessary commercial infrastructure at such time as we believe that eteplirsen is approaching marketing approval. We will continue to evaluate whether to market our DMD product candidates outside of the United States ourselves or enter into arrangements with other pharmaceutical or biotechnology companies for the marketing and sale of our products outside the United States either globally or on a country-by-country basis.

Table of Contents

Patents and Proprietary Rights

Our success depends in part upon our ability to protect our core technology and intellectual property. To accomplish this, we rely on a combination of intellectual property rights, including patents, trade secrets, copyrights and trademarks, as well as regulatory exclusivity and contractual protections.

We seek patent protection for certain of our proprietary technologies by filing patent applications in the United States and other countries. As of February 28, 2014, we owned or controlled approximately 312 U.S. and corresponding foreign patents and 186 U.S. and corresponding foreign patent applications. We intend to protect our proprietary technology with additional filings as appropriate.

Our patents and patent applications are directed to our product candidates as well as to our RNA-based technology platforms. Although we believe our patents and patent applications provide us with a competitive advantage, the ability to fully realize the potential for exclusivity based on our patent positions can be uncertain as they typically involve complex legal and factual questions. Even if patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by or licensed to us or our collaborators. Even if our patents and patent applications do provide our product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product candidates or platform technology due to patent positions held by a third party. For example, our competitor Prosensa has rights to European Patent No. EP 1619249. We opposed this patent 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 appealed this decision in June 2013; however, pending final resolution of this matter, the patent at issue may provide the basis for Prosensa or other parties that have rights to such patent to assert that our drug eteplirsen infringes on such patent. The outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal in the European Union we are unsuccessful in invalidating Prosensa s claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both eteplirsen and other therapeutic candidates for our pan-exon strategy could be materially impaired. We are also aware of certain pending and granted claims that have been issued to Prosensa in Japan that may provide the basis for Prosensa or other parties to assert that eteplirsen 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 any such proceeding cannot be predicted or determined as of the date of this report. We are also aware of certain claims that Prosensa has rights to in the United States that may provide the basis for Prosensa or other parties that have rights to these claims to assert that our drug eteplirsen infringes on such claims. We believe we have valid defenses to any such allegations or a basis to invalidate some or all of these claims and do not believe that Prosensa s granted claims and pending claims should they be granted should be deemed to prevent our ability to commercialize eteplirsen in the United States; however, as noted below, the biotechnology intellectual property landscape continues to evolve and we cannot be certain of this assessment. The DMD patent landscape is continually evolving and multiple parties, both commercial entities and academic institutions, may have rights to claims that could provide these parties a basis to assert that our product candidates infringe on these claims. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. We cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

Our product candidates and our technology are primarily protected by composition of matter and use patents and patent applications. Currently, our lead clinical product candidates are AVI-7288 (Marburg), AVI-7100 (Influenza) and AVI-4658 (Eteplirsen). We own issued patents covering composition and methods of use for AVI-7288 in the United States. We have exclusively licensed patents covering composition of matter and methods of use for AVI-4658 in the United States and Europe. Additionally, we have pending patent applications

Table of Contents

for composition and methods of use for AVI-7100 and issued and/or pending patent applications for composition and methods of use for other product candidates in the United States, Canada, South America, Europe, Asia, Australia, New Zealand, and/or the Middle East. Patent protection based on currently granted patents and patents granting from currently pending patent applications covering our product candidates and our technology will expire over the following time frames:

Product Candidate / Technology	Expiration of Patent Protection*
Eteplirsen	2025 (patents) 2030 (patents)
Other DMD exons	2025 (patents) 2034 (patent applications)
Exon-skipping	2013 (patents) 2023 (patents)
Antivirals (Ebola, Marburg, Dengue and Influenza)	2022 (patents) 2030 (patents)
Chemistry (PPMO, PMO*plus*® and PMO-X)	2024 (patents) 2032 (patent applications)
Antibacterials	2018 (patents) 2031 (patent applications)
Other rare diseases	2025 (patent applications) 2034 (patent applications)
Other targets and programs	2019 (patents) 2034 (patent applications)

* Stated expiration dates do not account for any patent term extension or pediatric extensions that may be available in the United States and certain foreign jurisdictions.

In addition to patent protection, we also rely on trade secrets and proprietary know-how, especially when we do not believe that patent protection is appropriate or can be obtained. Our policy is to require each of our employees, consultants and advisors to execute a confidentiality and inventions assignment agreement before beginning their employment, consulting or advisory relationship with us. These agreements generally provide that the individual must keep confidential and not disclose to other parties any confidential information developed or learned by the individual during the course of their relationship with us except in limited circumstances. These agreements also generally provide that we shall own all inventions conceived by the individual in the course of rendering services to us.

We are the owner of four federal trademark registrations in the United States: AVI BioPharma®, Cytoporter®, PMO-plus® and NeuGene®. We have pending trademark applications in the United States for the following trademarks: PMO-X ; Sarepta ; Sarepta Therapeutics and the Sarepta Therapeutics logo; Let s Skip Ahead ; The Promise of Science, Realized ; Transformation, Within Reach ; and Turning Discovery Into Recovery . In the European Union, we have trademark registrations for AVI BioPharma® and Sarepta® and pending applications for the following trademarks: the Sarepta Therapeutics logo; The Promise of Science, Realized ; Transformation, Within Reach ; and Turning Discovery Into Recovery . We have licensed certain technology to supplement and support certain of our core technologies. We have certain obligations and minimum royalties under those agreements, which costs are not material to our business and can be terminated at our discretion with minimal notice.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of the use, formulation and structure of our product candidates, and the methods used to manufacture them, as well as successfully defending these patents against third-party challenges. Our ability to protect our product candidates from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

We do not have patents or patent applications in every jurisdiction where there is a potential commercial market for our product candidates. For each of our programs, our decision to seek patent protection in specific foreign markets, in addition to the United States, is based on many factors, including:

- our available resources;
- the number and types of patents already filed or pending;
- the likelihood of success of the product candidate;

Table of Contents

- the size of the commercial market;
- the presence of a potential competitor in the market; and
- whether the legal authorities in the market effectively enforce patent rights.

We continually evaluate our patent portfolio and patent strategy and believe our owned and licensed patents and patent applications provide us with a competitive advantage; however, if markets where we do not have patents or patent applications become commercially important, our business may be adversely affected.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the United States, and tests used for determining the patentability of patent claims in all technologies are in flux. In addition, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. For example, patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the United States is even more uncertain. Changes in either the patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in the patents that we own or have licensed or in third-party patents. Further, since publication of discoveries in scientific or patent literature often lags behind actual discoveries, there is no assurance that we were the first creator of inventions covered by our pending patent applications, or that we were the first to file patent applications for these inventions.

Government Regulation

The testing, manufacturing, labeling, advertising, promotion, distribution, exportion and marketing of our products are subject to extensive regulation by governmental authorities in the United States and in other countries. In the United States, the FDA, under the Federal Food, Drug and Cosmetic Act and its implementing regulations, regulates pharmaceutical products. Failure to comply with applicable U.S. requirements may subject us to administrative or judicial sanctions, such as FDA refusal to approve pending NDAs, withdrawal of approval of approved products, warning letters, untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, civil penalties and/or criminal prosecution.

Drug Approval Process

To obtain FDA approval of a product candidate, we must, among other things, submit data providing substantial evidence of safety and efficacy of the product, as well as detailed information on the manufacture and composition of the product candidate and proposed labeling. The testing and collection of data and the preparation of necessary applications are expensive and time-consuming. The FDA may not act quickly or favorably in reviewing these applications, and we may encounter significant difficulties or costs in our efforts to obtain FDA approvals that could delay or preclude us from marketing our products.

The steps required before a drug may be approved for marketing in the United States generally include the following, with exceptions noted in the section captioned Government Regulation Animal Rule :

- preclinical laboratory tests and animal tests;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials commence;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug product for each indication;

Table of Contents

the submission to the FDA of an NDA;

satisfactory completion of an FDA inspection of the manufacturing facilities at which the product is made to assess compliance with cGMP;

satisfactory FDA audit of the clinical trial site(s) that generated the data in support of the NDA and also potentially the nonclinical site(s); and

FDA review and approval of the NDA.

Preclinical studies may include laboratory evaluations of the product chemistry, toxicity, and formulation, as well as animal studies to assess the potential safety and efficacy of the product candidate. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the preclinical studies, together with manufacturing information and analytical data, are submitted to the FDA as part of the IND, which must become effective before clinical trials may be commenced. The IND will become effective automatically 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the conduct of the clinical trials as described in the protocol submitted as part of the IND prior to that time. In this case, the trials are placed on clinical hold, and the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can proceed.

Clinical trials involve the administration of the product candidate to healthy volunteers or participants under the supervision of a qualified principal investigator. Clinical trials are conducted under protocols detailing the objectives of the study, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted in accordance with the FDA s good clinical practices requirements and state subject rights laws. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. The IRB will consider, among other things, clinical trial design, participant informed consent, ethical factors, the safety of human subjects, and the possible liability of the institution. The FDA may order the partial, temporary or permanent discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial subjects. The IRB may also require the clinical trial at that site to be halted, either temporarily or permanently, for failure to comply with the IRB s requirements, or may impose other conditions.

Clinical trials typically are conducted in three sequential phases prior to approval, but the phases may overlap. A fourth, or post-approval, phase may include additional clinical studies. These phases generally include the following; however, in the rare disease space, the number of subjects involved in each phase can be significantly less than the general parameters set forth below:

Phase I. Phase I clinical trials involve the initial introduction of the drug into human subjects. These studies are designed to determine the safety of usually single doses of the compound and determine any dose limiting intolerance, as well as evidence of the metabolism and pharmacokinetics of the drug in humans. Phase I studies usually involve less than 100 subjects and are most commonly conducted in healthy adult volunteers.

Phase II. Phase II clinical trials usually involve studies in a limited patient population to evaluate the efficacy of the drug for specific, targeted indications, to determine dosage tolerance and optimal dosage, and to identify possible adverse effects and safety risks. Phase II studies usually involve patients with the disease under investigation and numbers may vary from several dozen to several hundred.

Phase III. If a compound is found to be potentially effective and to have an acceptable safety profile in Phase II (or sometimes Phase I) studies, the clinical trial program will be expanded to further confirm clinical efficacy, optimal dosage and safety within an expanded patient population which may involve

Table of Contents

geographically dispersed clinical trial sites. Phase III studies usually include several hundred to several thousand patients. Generally, two adequate and well-controlled Phase III clinical trials are required by the FDA for approval of an NDA.

Phase IV. Phase IV clinical trials are studies required of or agreed to by a sponsor that are conducted after the FDA has approved a product for marketing. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of drugs approved under accelerated approval regulations. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase IV clinical trial requirement. Failure to promptly conduct Phase IV clinical trials could result in withdrawal of approval for products approved under accelerated approval regulations.

A company seeking marketing approval for a new drug in the United States must submit to the FDA the results of the preclinical and clinical trials, together with, among other things, detailed information on the manufacture and composition of the product candidate and proposed labeling, in the form of an NDA, including payment of a user fee. The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has ten months in which to complete its initial review of a standard NDA and respond to the applicant, and six months for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the NDA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date. If the FDA s evaluations of the NDA and the clinical and manufacturing procedures and facilities are favorable, the FDA may issue an approval letter. If the FDA finds deficiencies in the NDA, it may issue a complete response letter, which contains the conditions that must be met in order to secure final approval of the NDA. If and when those conditions have been met to the FDA s satisfaction, the FDA will issue an approval letter, authorizing commercial marketing of the drug for certain indications. If the FDA s evaluation of the NDA submission and the clinical and manufacturing procedures and facilities is not favorable, the FDA may refuse to approve the NDA. Sponsors that receive a complete response letter may submit to the FDA information that represents a complete response to the issues identified by the FDA. Resubmissions by the NDA sponsor in response to a complete response letter trigger new review periods of varying length (typically two to six months) based on the content of the resubmission. The FDA may also refer an application to an appropriate advisory committee, typically a panel of clinicians, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendations of the advisory committee.

A sponsor may also seek approval of its drug candidates under programs designed to accelerate the FDA s review and approval of NDAs. For instance, a sponsor may seek FDA designation of a drug candidate as a fast track product. Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate early, frequent, communication and begin reviewing sections of an NDA before the application is complete. This rolling review is available if the applicant provides, and the FDA approves, a schedule for the remaining information. We were granted fast track status for eteplirsen in 2007 and we announced in September 2012 that the FDA granted fast track status for the development of both AVI-7288 and AVI-7537.

The Food and Drug Administration Safety and Innovation Act, or FDASIA, enacted and signed into law in 2012 amended the criteria for the fast track and accelerated approval pathways and, as a result, the pathways now share many common eligibility criteria. FDASIA provides both sponsor companies and the FDA greater flexibility with expedited regulatory mechanisms. The statute clarifies that a fast track product may be approved pursuant to an accelerated approval (Subpart H) or under the traditional approval process. In addition, FDASIA codified the accelerated approval pathway as separate and apart from fast track pathway, meaning that for drugs

Table of Contents

to be eligible for accelerated approval, they do not need to be designated under the fast track pathway. FDASIA reinforces the FDA s authority to grant accelerated approval based on surrogate endpoints that are reasonably likely to predict clinical benefit, and provides for a more expansive use of non-surrogate clinical endpoints by authorizing the FDA to grant accelerated approval based on the use of clinical endpoints that can be measured earlier in the development process than irreversible morbidity or mortality, and that are reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. In determining whether to grant accelerated approval, the FDA must consider the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Approvals of this kind typically include requirements for appropriate post-approval Phase IV clinical trials. FDASIA retains this requirement and further requires those studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit. We had various meetings with the FDA in 2013 and will continue to have meetings with the FDA during 2014 to discuss the clinical results from our Phase IIb study of eteplirsen and most appropriate regulatory review pathway based on these data. In addition, we also plan to have discussions with the FDA to finalize the pivotal clinical study design for eteplirsen. Based on feedback from these meetings, we will continue to pursue the most appropriate regulatory path for regulatory review and approval of eteplirsen. Our determination will be further informed by subsequent meetings with the FDA.

Additionally, FDASIA established a new, expedited regulatory mechanism referred to as breakthrough therapy designation. Breakthrough therapy designation, fast track, and accelerated approval are not mutually exclusive and are meant to serve different purposes. The breakthrough therapy designation is focused on expediting the development and review process and by itself does not create an alternate ground for product approval. A sponsor may seek FDA designation of a drug candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA issued draft guidance entitled Expedited Programs for Serious Conditions Drugs and Biologics in June 2013 and if deemed necessary, is required to amend its regulations by July 2014. We will continue to evaluate, with input from the FDA, which expedited programs are appropriate to incorporate in our regulatory approach for eteplirsen and our other DMD product candidates.

Finally, drug candidates, upon submission of an NDA, may also be eligible for priority review, or review within a six month timeframe from the date a complete NDA is accepted for filing, if a sponsor shows that its drug candidate provides a significant improvement compared to marketed drugs.

While FDASIA provides certain authorities and direction to the FDA, it is unclear how the FDA will interpret and implement FDASIA provisions, in particular, in considering what the appropriate regulatory approval pathway is for eteplirsen. We cannot be sure that any of our drug candidates will qualify for any of these expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review.

Often, even after a drug has been approved by the FDA for sale, the FDA may require that certain post-approval requirements be satisfied, including the conduct of additional clinical studies. If such post-approval conditions are not satisfied, the FDA may withdraw its approval of the drug. In addition, holders of an approved NDA are required to:

- report certain adverse reactions to the FDA;
- submit annual and periodic reports summarizing product information and safety data;
- comply with certain requirements concerning advertising and promotional labeling for their products; and
- continue to have quality control and manufacturing procedures conform to cGMP after approval.

Table of Contents

The FDA periodically inspects the sponsor s records related to safety reporting and/or manufacturing; this latter effort includes assessment of compliance with cGMP. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved NDA, including withdrawal of the product from the market.

Many other countries and jurisdictions have similar drug development and regulatory review processes. We have conducted clinical trials in the United Kingdom and intend to submit for marketing approval in countries other than the United States. Therefore, we will have to comply with the legal and regulatory requirements in the countries where we conduct trials and submit for marketing approval.

Animal Rule

In the case of product candidates that are intended to treat rare life-threatening diseases, such as infection caused by exposure to various hemorrhagic fever viruses, conducting controlled clinical trials to determine efficacy may be unethical or unfeasible. Under regulations issued by the FDA in 2002, often referred to as the Animal Rule, the approval of such products can be based on clinical data from trials in healthy human subjects that demonstrate adequate safety, and immunogenicity and efficacy data from adequate and well-controlled animal studies. Among other requirements, the animal studies must establish that the drug or biological product is reasonably likely to produce clinical benefits in humans. Because the FDA must agree that data derived from animal studies may be extrapolated to establish safety and effectiveness in humans, seeking approval under the Animal Rule adds significant time, complexity and uncertainty to the testing and approval process. No animal model is established as predicting human outcomes in the prevention or treatment of any filovirus disease. We have yet to demonstrate the predictive value of our animal studies to the FDA s satisfaction. In addition, products approved under the Animal Rule are subject to additional requirements including post-marketing study requirements, restrictions imposed on marketing or distribution or requirements to provide information to patients. The Animal Rule is a rarely-used regulatory pathway and most of the products approved to-date under the Animal Rule have built upon existing indications with human data to support efficacy. Additional clarity on Animal Rule requirements is not anticipated until the FDA releases an updated version of its draft guidance on the Animal Rule that was first published in January 2009.

Emergency Use Authorization

The Commissioner of the FDA, under delegated authority from the Secretary of DHHS may, under certain circumstances, issue an Emergency Use Authorization, or EUA, that would permit the use of an unapproved drug product or unapproved use of an approved drug product. Before an EUA may be issued, the Secretary must declare an emergency based on one of the following grounds:

- a determination by the Secretary of the Department of Homeland Security that there is a domestic emergency, or a significant potential for a domestic emergency, involving a heightened risk of attack with a specified biological, chemical, radiological or nuclear agent or agents;
- a determination by the Secretary of DoD that there is a military emergency, or a significant potential for a military emergency, involving a heightened risk to U.S. military forces of attack with a specified biological, chemical, radiological, or nuclear agent or agents; or
- a determination by the Secretary of DHHS of a public health emergency that effects or has the significant potential to affect, national security, and that involves a specified biological, chemical, radiological, or nuclear agent or agents, or a specified disease or condition that may be attributable to such agent or agents.

In order to be the subject of an EUA, the FDA Commissioner must conclude that, based on the totality of scientific evidence available, it is reasonable to believe that the product may be effective in diagnosing, treating, or preventing a disease attributable to the agents described above; that the product s potential benefits outweigh its potential risks; and that there is no adequate, approved alternative to the product.

Table of Contents

The Pandemic and All-Hazards Preparedness Reauthorization Act of 2013 (PAHPRA) enhanced existing EUA requirements, including by:

- Providing clearer authority for the FDA to issue EUAs before a chemical, biological, radiological or nuclear emergency occurs to enable stakeholders to prepare for use of unapproved medical products, or unapproved uses of approved products, if certain criteria are met (referred to as pre-event EUAs)
- Allowing the FDA to issue an EUA based on the HHS Secretary s determination that there is a potential for a public health emergency involving a chemical, biological, radiological or nuclear threat agent (not only based on an actual emergency)
- Expanding the time period for collection and analysis of information about a medical countermeasure s safety and effectiveness for a reasonable period beyond the effective period of the EUA
- Expressly permitting FDA, as part of issuance of an EUA, to categorize the complexity of an in vitro diagnostic device to indicate whether the test can be performed at a point-of-care setting or only in a more sophisticated laboratory

The FDA strongly encourages an entity with a possible candidate product, particularly one at an advanced stage of development, to contact the FDA Center responsible for the candidate product before a determination of actual or potential emergency. Such an entity may submit a request for consideration that includes data to demonstrate that, based on the totality of scientific evidence available, it is reasonable to believe that the product may be effective in diagnosing, treating, or preventing the serious or life-threatening disease or condition. This is called a pre-EUA submission and its purpose is to allow FDA review considering that during an emergency, the time available for the submission and review of an EUA request may be severely limited. We intend to work with DoD in the future on a pre-EUA submission with respect to our product candidate intended to treat Marburg in order to inform and expedite the FDA s issuance of an EUA, should one become necessary in the event of an emergency or potential emergency.

Orphan Drug Designation and Exclusivity

In the United States, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. In the United States, orphan drug designation must be requested before submitting an application for marketing approval. An orphan drug designation does not shorten the duration of the regulatory review and approval process. The approval of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and efficacy of a compound must be established through adequate and well-controlled studies. If a product which has an orphan drug designation subsequently receives FDA approval for the indication for which it has such designation, the product is entitled to an orphan drug exclusivity period, which means the FDA may not grant approval to any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as where an alternative product demonstrates clinical superiority to the product with orphan exclusivity. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of marketing exclusivity for the drug. Distinct from orphan drug exclusivity, the FDA may provide six months of pediatric exclusivity to a sponsor of an NDA, if the sponsor conducted a pediatric study or studies of such product. This process is initiated by the FDA as a written request for pediatric studies that applies to sponsor s product. If the sponsor conducts qualifying studies and the studies are accepted by the FDA, then an additional six months of pediatric exclusivity will attach to any periods of regulatory exclusivity such as orphan drug exclusivity and new chemical entity exclusivity, and any Orange Book listed patents for the listed product. Competitors may receive approval of different drugs or biologics for the indications for which a prior approved orphan drug has exclusivity. We have been granted orphan drug designation for eteplirsen, AVI-7288, AVI-7537 and AVI-5038 in the United States.

Table of Contents

The European Orphan Drug Regulation as applied by the European Medicines Agency (EMA) is considered for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition afflicting five or fewer out of 10,000 people in the European Union, including compounds that for serious and chronic conditions would likely not be marketed without incentives due to low market return on the sponsor s development investment. The medicinal product considered should be of significant benefit to those affected by the condition as compared to previously approved products for the same indication. Benefits of being granted orphan drug designation are significant, including ten years of market exclusivity. During this ten-year period, the EMA may not accept a new marketing application for a similar drug for the same therapeutic indication as the orphan drug. Distinct from orphan drug exclusivity, the EMA may provide a sponsor having an approved Pediatric Investigation Plan (PIP) or pediatric exclusivity waiver, which may lead to a two-year period of market exclusivity beyond the original ten-year period of orphan drug exclusivity. We have been granted orphan drug designation for eteplirsen and AVI-5038 in the European Union.

Other Regulatory Requirements

In addition to regulation by the FDA and certain state regulatory agencies, we are also subject to a variety of foreign regulations governing clinical trials and the marketing of other products. Outside of the United States, our ability to market a product depends upon receiving a marketing authorization from the appropriate regulatory authorities. The requirements governing the conduct of clinical trials, marketing authorization, pricing and reimbursement vary widely from country to country. In any country, however, we will only be permitted to commercialize our products if the appropriate regulatory authority is satisfied that we have presented adequate evidence of safety, quality and efficacy. Whether or not FDA approval has been obtained, approval of a product by the comparable regulatory authorities of foreign countries must be obtained prior to the commencement of marketing of the product in those countries. The time needed to secure approval may be longer or shorter than that required for FDA approval. The regulatory approval and oversight process in other countries includes all of the risks associated with regulation by the FDA and certain state regulatory agencies as described above.

Pharmaceutical Pricing and Reimbursement

In both U.S. and foreign markets, our ability to commercialize our products successfully, and to attract commercialization partners for our products, depends in significant part on the availability of adequate financial coverage and reimbursement from third-party payers, including, in the United States, governmental payers such as the Medicare and Medicaid programs, managed care organizations, and private health insurers. Third-party payers are increasingly challenging the prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost effectiveness of our products. Even with the availability of such studies, our products may be considered less safe, less effective or less cost-effective than alternative products, and third-party payers may not provide coverage and reimbursement for our product candidates, in whole or in part.

Political, economic and regulatory influences are subjecting the healthcare industry in the United States to fundamental changes. There have been, and we expect there will continue to be, legislative and regulatory proposals to change the healthcare system in ways that could significantly affect our business, including the Patient Protection and Affordable Care Act of 2010. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures include:

controls on government funded reimbursement for drugs;

mandatory discounts under certain government sponsored programs;

controls on healthcare providers;

challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;

Table of Contents

reform of drug importation laws; and

expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted could have a material adverse effect on our business prospects.

Competition

The pharmaceutical and biotechnology industries are intensely competitive, and any product candidate developed by us would likely compete with existing drugs and therapies. There are many pharmaceutical companies, biotechnology companies, public and private universities, government agencies and research organizations that compete with us in developing various approaches to the treatment of rare and infectious diseases. Many of these organizations have substantially greater financial, technical, manufacturing and marketing resources than we have. Several of them have developed or are developing therapies that could be used for treatment of the same diseases that we are targeting. In addition, many of these competitors have significantly greater commercial infrastructures than we have. Our ability to compete successfully will depend largely on:

our ability to complete clinical development and obtain regulatory approvals for our product candidates;

the efficacy, safety and reliability of our product candidates;

the timing and scope of regulatory approvals;

product acceptance by physicians and other health-care providers;

protection of our proprietary rights and the level of generic competition;

the speed at which we develop product candidates;

our ability to supply commercial quantities of a product to the market;

obtaining reimbursement for product use in approved indications;

our ability to recruit and retain skilled employees; and

the availability of substantial capital resources to fund development and commercialization activities, including the availability of funding from the U.S. government.

DMD Program Competition. Currently, no product has been approved for the treatment of DMD. Companies including, but not limited to, Prosensa (which announced it regained rights to Drisaparsen and all other programs for the treatment of DMD from GlaxoSmithKline plc, or

GSK, in January 2014), and PTC Therapeutics, Inc., or PTC, have product candidates in development for the treatment of DMD. PTC has an exon 51 skipping product candidate in early development while Prosensa is reviewing its Phase III data to determine whether it can seek approval of its exon 51 skipping product candidate drisaparsen. Several companies have recently entered into collaborations or other agreements for the development of product candidates, including messenger RNA, gene or small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including Biogen Idec, Inc., Isis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., PTC, Sanofi, Alnylam Pharmaceuticals, Inc., or Alnylam, Moderna Therapeutics, Inc., Summit plc and Oxford University.

The Prosensa / GSK program commenced treatment in December 2010 in a Phase III clinical study in ambulant individuals with DMD who have a dystrophin gene mutation amenable to treatment by skipping

Table of Contents

exon 51. Prosensa s candidate for skipping exon 51, GSK2402968, utilizes a different chemistry, 2 O-methyl-phosphorothioate, which has the potential for different performance, safety and tolerability characteristics than eteplirsen. This randomized, placebo-controlled study was fully enrolled, with approximately 180 participants who were being dosed for 48 weeks. The primary efficacy endpoint for Prosensa s study was a measure of muscle function using the 6-MWT. In September 2013, GlaxoSmithKline plc and Prosensa announced that the Phase III clinical study of drisaparsen did not meet the primary endpoint of a statistical significant improvement in the 6 MWT compared to placebo. In September 2010, the Prosensa / GSK program commenced a Phase II double-blind, placebo-controlled study. This study is designed to assess the efficacy of two different dosing regimens of GSK2402968 administered over 24 weeks in DMD patients, and then to continue observing the patients over a second 24-week interval for a total study time frame of 48 weeks. This study completed enrollment with 54 DMD patients in October 2011 and has since concluded. Another study using GSK2402968 in non-ambulatory DMD patients has been initiated using a 6 mg/kg dose and is anticipated to enroll 20 patients. Like Prosensa, other companies continue to pursue approval of products for the treatment of DMD and their products may or may not prove to be safer and/or more efficacious than, or obtain marketing approval before, eteplirsen.

Hemorrhagic Fever Virus Programs. No specific treatment has been proven effective, and no approved vaccine currently exists for either Ebola or Marburg. Investigational compounds cannot be tested for efficacy on humans except in outbreak environments so these agents must be tested extensively in animals and meet strict government regulations. Vaccine development is in the early stages in both the biotechnology industry and by U.S. government agencies (*e.g.*, the National Institute of Allergy and Infectious Diseases and the DoD). The government is also supporting early stage research on therapeutics against hemorrhagic fever viruses, including broad-spectrum therapeutics. With respect to therapeutics in advanced development, on January 2014, Tekmira Pharmaceuticals Corp. announced it has dosed the first subject in aPhase I human clinical trial for TKM-Ebola, a systemically delivered RNAi therapeutic for the treatment of Ebola virus infection under a contract with the DoD. We commenced initial human safety studies of our therapeutic candidates against Marburg and Ebola viruses in May 2011, however, the DoD terminated further funding of our Ebola program in October 2012 and further development of our Marburg therapeutic candidate will depend on receiving additional funding from the DoD.

Influenza Program. Currently, there are two therapeutic products for influenza that have received market approval from the FDA and are recommended for use in the United States. These are: (1) oseltamivir (Tamiflu), a Roche Holding and Gilead product; and (2) zanamivir (Relenza), a GSK product. In addition to these products, Biota Pharmaceuticals and Daiichi Sankyo s laninamivir and BioCryst s peramivir were launched in 2010 in Japan. Currently, funding from the DHHS Biomedical Advanced Research and Development Authority is helping support clinical trials of Biota and Daiichi Sankyo s laninamivir, BioCryst s peramivir, Ansun Biopharma s Fludase, and Romark Laboratories nitazoxanide. In addition, other companies have influenza therapeutic compounds against viral and host targets in various stages of development, including Toyama Chemical s favipiravir which is in a Phase II clinical trial in the United States, under a DoD contract with MediVector, Inc., and has completed a Phase III trial in Japan. DHHS is currently seeking additional antiviral therapeutics for the treatment of influenza infections.

In addition to therapeutic products, other companies are focusing development efforts on universal influenza vaccines, including BiondVax Pharmaceuticals Ltd. and Immune Targeting Systems which are in Phase II and Dynavax in Phase I. Successful development of a universal influenza vaccine could lead to a reduction in the number of influenza cases and, therefore, the market size.

Platform Technology. We believe that other biotechnology and pharmaceutical companies share a focus on RNA-based drug discovery and development. Competitors with respect to our RNA-based technologies include, but are not limited to, Alnylam, Tekmira Pharmaceuticals Corp., Isis, Prosensa, Sanofi Aventis, and Santaris Pharma A/S. We are unaware of any other commercial organization that is developing therapeutics based on a PMO chemistry platform.

Table of Contents

Research and Development

Since our inception, we have focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. We are primarily focused on rapidly advancing the development of our potentially disease-modifying DMD drug candidates, including our lead product candidate, eteplirsen. We are also focused on developing therapeutics for the treatment of infectious diseases, including our lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus. By building our infectious disease programs, which are primarily funded and supported by the DoD, and leveraging our highly-differentiated, proprietary technology platforms including our next-generation PMO chemistries (PMO-X , PMO-*plus*®, and PPMO) which we have designed to enhance delivery, target selectivity and drug potency, we are seeking to further develop our research and development competencies and identify additional product candidates.

During 2012, we completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial. We are working with the FDA to initiate a pivotal clinical study and determine the feasibility of expedited regulatory programs for eteplirsen. The lengthy process of securing FDA approvals for new drugs requires the expenditure of substantial resources. Accordingly, we cannot currently estimate, with any degree of certainty, the amount of time or money that we will be required to expend in the future on our product candidates prior to their regulatory approval, if such approval is ever granted.

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

The following table summarizes the primary components of our research and development external expenditures for our principal research and development programs, and our internal research and development expenditures in the aggregate for each of the years ended December 31, 2013, 2012 and 2011. Prior to January 1, 2011, the Company did not track research and development expenditures on a project level, as such, the inception-to-date expenses represent the period from January 1, 2011 to December 31, 2013.

	Year Ended December 31,			January 1, 2011 to December 31,
	2013	2012	2011	2013
Research and Development Expense (in thousands)				
Development programs				
DMD	$ 43,511	$ 12,181	$ 10,420	$ 66,112
Infectious Diseases	5,701	22,956	28,016	56,673
Internal research and development costs	23,697	17,265	28,426	
Total research and development expense	$ 72,909	$ 52,402	$ 66,862	

Employees

As of December 31, 2013, we had 146 employees, 54 of which hold advanced degrees. Of these employees, 98 are engaged directly in research and development activities and 48 are in administration. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good.

Table of Contents

Item 1A. Risk Factors.

Factors That Could Affect Future Results

Set forth below and elsewhere in this Annual Report on Form 10-K 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 Annual Report on Form 10-K. Please review our legend titled Forward-Looking Information at the beginning of this Annual Report on Form 10-K which is incorporated herein by reference. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Relating to Our Business

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

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

Our ability to commercialize any of our product candidates, including eteplirsen, depends on first receiving required regulatory approvals. It is possible that our product candidates, including eteplirsen, may never receive regulatory approval, including any designations that would expedite the review or approval process for various reasons, including: any failure to meet the applicable regulatory requirements to obtain regulatory approval for any of our product candidates including any failure to conduct a pivotal study with an FDA approved design, file an NDA prior to or in the time-frame suggested by the FDA, demonstrate the safety and effectiveness for any of our product candidates, lack of funding, changes in the regulatory landscape, new scientific developments, including the results for clinical trials of competitor drugs, and the FDA s interpretation and analysis of such developments in connection with our product candidates, manufacturing or other reasons. If we are unable to obtain regulatory approval for any of our current product candidates, it could delay or eliminate any potential product commercialization and product revenue for our Company.

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

- 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;

Table of Contents

the product s potential advantage over alternative treatment methods;

whether the product can be produced in commercial quantities and at acceptable costs;

marketing and distribution support for the product; and

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

We have been granted orphan drug status for certain of our product candidates, but there can be no guarantee that we will be able to prevent third parties from developing and commercializing products that are competitive to these product candidates.

To date we have been granted orphan status under the Orphan Drug Act by the FDA for: two of our product candidates in DMD (including eteplirsen), AVI-6002 and AVI-7537 for the treatment of Ebola virus and AVI-6003 and AVI-7288 for the treatment of Marburg virus. Generally, product candidates granted orphan status are provided with seven years of marketing exclusivity by the FDA upon New Drug Application (NDA) approval, meaning the FDA will generally not approve applications for product candidates that contain the same active ingredient and are labeled for the same orphan indication. Even if we are the first to obtain marketing exclusivity through an approval of an orphan product in the United States, there are limited circumstances under which a later product from a competitor may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our product or due to an inability to assure a sufficient quantity of the orphan drug.

To date we have been granted orphan drug medicinal product designations in the European Union for our lead drug candidate, eteplirsen, and AVI-5038 for the treatment of DMD. Product candidates granted orphan status in Europe can be provided with up to 10 years of marketing exclusivity, meaning that another application for marketing authorization of a later similar medicinal product for the same therapeutic indication will generally not be approved in Europe. Pediatric product candidates may be eligible for an additional two years of marketing exclusivity. Although we may have drug candidates that have or may obtain orphan drug exclusivity in Europe, the orphan designation and associated exclusivity period may be modified for several reasons, including the designation criteria may have significantly changed since market authorization of the orphan product, (e.g., product profitability exceeds the criteria for orphan drug designation), there are production or supply problems with the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

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

Table of Contents

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

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

Further, the FDA and other foreign regulatory agencies have substantial discretion in the approval process, and the determination of when or whether regulatory approval, of any type, will be granted for any product candidate we develop. In this regard, even if we believe data collected from clinical trials of our product candidates are promising and our CMC and related manufacturing processes are satisfactory, the FDA or foreign authorities may disagree with our interpretations and determine such data is not sufficient to accept our application or support approval. Furthermore, the FDA or other foreign regulatory agencies may approve a product candidate for fewer indications than requested or may grant approval subject to the performance of post-approval or confirmatory studies for a product candidate. Similarly, the FDA or other foreign regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

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

Table of Contents

candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain regulatory approval, which could delay or eliminate any potential commercialization or product revenue for any of our product candidates.

We continue to work with the FDA in our pursuit to obtain FDA approval of eteplirsen. The Company believes that the new issues and concerns raised by the FDA in recent meetings with the Company may result in delays in commencing the Company s pivotal clinical study as well as its filing of an NDA for eteplirsen. The Company does not currently have enough information at this time to determine the length of such delays, although we are working towards beginning an eteplirsen pivotal clinical study and dosing the first patient in the second or third quarter of 2014. Furthermore, there can be no assurance that any submission or application will be accepted by the FDA (*e.g.*, refusal to file) or that any expedited or regular development, review or approval will be granted on a timely basis, or at all. The FDA or other foreign authorities could also request additional information or meetings with us or require us to conduct further studies or CMC-related work (*e.g.*, a complete response letter) prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for eteplirsen or any of our other product candidates would result in a longer time period for commercialization of such product candidate, could potentially increase the cost of development of such product candidate, could have a material adverse effect on our financial condition and could harm our competitive position in the marketplace.

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

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

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive preclinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future preclinical and clinical trials of our product candidates may not show sufficient safety or efficacy to obtain regulatory approvals.

For example, in 2012, we completed Study 201, a U.S.-based Phase IIb 12 person clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. Following completion of this study, we initiated Study 202, an ongoing open label extension study with the same participants from Study 201. These trials were initiated, in part, to further demonstrate efficacy and safety, including the production of dystrophin, and explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. While Studies 201 and 202 met their primary endpoints at weeks 24 and 48 respectively, and results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot assure you that data from the ongoing open label

Table of Contents

extension study will be sufficient for regulatory approval or will continue to be positive through the remaining study period. If these extension data are not sufficient to demonstrate safety and efficacy to regulators, do not continue to demonstrate safety and efficacy through the remainder of Study 202, or are insufficient to identify a consistently effective dose, we expect we will need to make progress in our discussions with regulatory authorities about the design and subsequent execution of any further studies that may be required. Regulatory authorities might require more extensive information or preclinical or clinical trials than anticipated. Such clinical trials might include additional open label extension studies for all participants, who have previously received eteplirsen, as well as other participants (*e.g.*, non-ambulatory participants), additional placebo-controlled pivotal or confirmatory study or studies, or other additional trials before conducting a pivotal clinical study of the product candidate. Any additional requirements for regulatory approval would increase our costs and delay submissions, studies and commercialization of eteplirsen. We may not be able to, or it may be difficult for us to conform to regulatory guidance, including related to clinical trial design and the timing of NDA filings, and even if we conform to any guidance regulatory authorities provide, it does not guarantee receipt of marketing approval, even if we believe our preclinical and clinical trials are successful.

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

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

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

Our product candidates require precise, high-quality manufacturing. The failure to achieve and maintain high quality standards, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in patient injury or death or product recalls. Contract drug manufacturers often encounter difficulties involving production yields, quality control and quality assurance and shortages of qualified personnel. If our contract manufacturers or other third parties fail to deliver our product candidates for our research and development programs, clinical use or potential commercial supply on a timely basis, with sufficient quality, and at commercially reasonable prices, and we fail to find replacement manufacturers or to develop our own

Table of Contents

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

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

Our contract manufacturers are required to produce our clinical product candidates under current Good Manufacturing Practice (cGMP) conditions in order to meet acceptable standards for our clinical trials. If such standards change, the ability of contract manufacturers to produce our product candidates on the schedule we require for our clinical trials may be affected. In addition, contract manufacturers may not perform their agreements with us or may discontinue their business before the time required by us to successfully produce and market our product candidates. We and our contract manufacturers are subject to periodic unannounced inspection by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations and corresponding foreign standards. We do not have control over a third-party manufacturer s compliance with these regulations and standards. Any difficulties or delays in our contractors manufacturing and supply of product candidates or any failure of our contractors to maintain compliance with the applicable regulations and standards could increase our costs, make us postpone or cancel clinical trials, prevent or delay regulatory approval by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, or cause our products to be recalled or withdrawn.

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

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for later stage clinical trials in eteplirsen and potential commercialization, we are working to increase the scale of production of our drug product in 2014. During 2014, we will also continue to evaluate whether and when to increase API production capacity which will depend in significant part on feedback from the FDA and our expectations regarding if and when we would commence a pivotal clinical study for eteplirsen and any subsequent commercialization. In order to conduct larger or late-stage scale clinical trials for a product candidate and supply sufficient quantities of the resulting drug product and its components, if that product candidate is approved for sale, we will need to manufacture it in larger quantities. We may not be able to successfully increase the manufacturing capacity for any of our product candidates, whether in collaboration with third-party manufacturers or on our own, in a manner that is timely, safe, compliant with cGMP or other applicable legal or regulatory standards or cost-effective 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,

Table of Contents

the intellectual property rights to those improvements. Significant scale-up of manufacturing may require additional processes, technologies and validation studies, which are costly, may not be successful and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a product candidate itself or of a product candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

In addition, in order to release product and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate our analytical methods or demonstrate adequate purity, stability or comparability of the product candidates in a timely or cost-effective manner or at all. If we are unable to successfully validate our analytical methods or to demonstrate adequate purity, stability, or comparability, the development of our product candidates and regulatory approval or commercial launch for any resulting drug products may be delayed, which could significantly harm our business.

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

We rely on U.S. government contracts and awards to fund and support certain development programs, including the Marburg program which accounts for substantially all of our current revenue. The funding of U.S. government programs is subject to Congressional appropriations. Congress generally appropriates funds on a fiscal year basis even though a program may extend over several fiscal years, as is the case with our DoD contract for the development of our Marburg product candidate. Consequently, programs are often only partially funded initially and additional funds are committed only as Congress makes further appropriations. If appropriations for one of our programs become unavailable, or are reduced or delayed, our contracts may be terminated or adjusted by the U.S. 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 U.S. government procurement due to lack of funding and such delays can affect our operations during the period of delay. The DoD operated under such a continuing resolution for the U.S. government s fiscal year 2013. Additionally, on March 1, 2013, a sequestration went into effect which implements across-the-board cuts to U.S. government agencies, totaling $1.2 trillion over 10 years. These cuts are to be split 50-50 between domestic and defense discretionary spending. The DoD had to make $47 billion in cuts before September 30, 2013. While Congress struck a two-year budget deal to provide sequester relief in 2014 and 2015, the deal lessens the cuts but does not cancel the sequestration. These and other potential budget cuts by the U.S. government as well as the effects of U.S. government shutdowns could have widespread ramifications including on the DoD s procurement and research and development programs. Sequestration may result in a reduction of funds available for new procurements, but existing contracts may also be reduced in scope, terminated, or partially terminated. The Department of Health and Human Services (DHHS) Special Reserve Fund, a $5.6 billion advanced appropriation to be used over 10 years to purchase medical countermeasures, expired at the end of the 2013 fiscal year. Going forward, Congress plans to replenish the Special Reserve Fund through the annual appropriations process introducing uncertainty with respect to availability of funding from year to year. As a result, the viability of the DHHS and its agencies as a partner and potential customer is uncertain.

In addition, U.S. government contracts generally also permit the U.S. government to terminate or renegotiate the contract, in whole or in part, without prior notice, at the U.S. government s convenience or for

Table of Contents

default based on performance. From time to time, we receive communications from the U.S. government regarding our performance, including requests for us to provide additional information and/or take certain steps to remedy noted deficiencies. While we work closely with our contacts at the U.S. government and believe we can adequately address issues raised through such communications, there is no guarantee that we will be able to adequately respond to all requests or remedy all deficiencies cited. If one of our contracts is terminated for convenience, we would generally be entitled to payments for our allowable costs and would receive some allowance for profit on the work performed. If one of our contracts is terminated for default, we would generally be entitled to payments for our work that has been completed to that point. A termination arising out of our default could expose us to liability and have a negative impact on our ability to obtain future contracts. Furthermore, if we fail to satisfy certain performance or deliverable requirements or to adhere to development timelines, revenues associated with the satisfaction of such requirements or timelines may be delayed or may not be realized.

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

Even if we successfully complete development of our Marburg and influenza product candidates, the major, if not only, potential purchaser is the U.S. government. The lack of a commercial market makes us reliant upon the U.S. government to determine and communicate the market for biodefense countermeasures and U.S. 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 including but not limited to those related to route of exposure, time to treat, and manufacturing demands. The FDA s requirements under the Animal Rule are also evolving which may result in additional studies being needed to characterize appropriate animal models. It is unclear whether funding will continue to be available to address evolving DoD and FDA requirements, and until future studies are completed, it is unclear whether our product candidates will successfully meet these requirements. If they do not, the DoD may choose to terminate the contract. Additionally, manufacturing demands may be such as to require enhancements to our manufacturing infrastructure, which DoD may not be able to fund through our existing research and development contract. With respect to the civilian sector, Marburg and influenza viruses are among the top public health threats, yet the broader demand for our product candidates remains uncertain.

This expected dependence on U.S. government purchases presents additional challenges, since the U.S. 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, the U.S. government can, in extreme cases, threaten compulsory licensing or purchase patent-breaching generics.

Table of Contents

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

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

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

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

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

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

We have completed a Phase Ib/II clinical trial for eteplirsen in the UK and announced results in October 2010, which were published in The Lancet in July 2011. We have also completed a U.S.-based Phase IIb placebo-controlled trial in eteplirsen and announced results in April 2012. Following completion of this study, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial and announced 48-week results on October 3, 2012, 62-week results on December 7, 2012, 74-week results on April 5, 2013, 84-week results on June 19, 2013, 96-week results on September 26, 2013 and 120-week results on January 15, 2014. We expect to commence additional trials of eteplirsen and other product candidates in the future based on feedback from the FDA. Each of our clinical trials requires the investment of substantial planning, expense and time, and the timing of the commencement, continuation and completion of

Table of Contents

these clinical trials may be subject to significant delays relating to various causes including new positions, issues and requests made by the FDA based on scientific developments and data from other drugs being developed by other companies for the treatment of diseases similar to or related to those targeted by our product candidates. Participant enrollment is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, the eligibility criteria for the trial, the existence of competing clinical trials and the availability of alternative or new treatments.

We depend on medical institutions and clinical research organizations (CROs), to conduct our clinical trials in compliance with Good Clinical Practice (GCP) and to the extent they fail to enroll participants for our clinical trials, fail to conduct the study to GCP standards or are delayed for a significant time in the execution of our trials, including achieving full enrollment, we may be affected by increased costs, program delays or both, which may harm our business. In addition, we have in the past conducted clinical trials in foreign countries and may do so again in the future, which may subject us to further delays and expenses as a result of increased drug shipment costs, additional regulatory requirements and the engagement of foreign CROs, as well as expose us to risks associated with less experienced clinical investigators who are unknown to the FDA, and different standards of medical care. Foreign currency transactions insofar as changes in the relative value of the U.S. dollar to the foreign currency where the trial is being conducted may impact our actual costs. In addition, for some programs, such as DMD and Marburg infection, there are currently no approved drugs to compare against and an agreement about how to measure efficacy has yet to be reached with the FDA and then demonstrated.

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

- Scientific developments and data available for investigational drugs being developed by third parties for the treatment of the same, similar or related diseases to those targeted by our product candidates;
- deficiencies in the trial design;
- deficiencies in the conduct of the clinical trial including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- deficiencies in the clinical trial operations or trial sites resulting in the imposition of a clinical hold;
- the product candidate may have unforeseen adverse side effects, including fatalities, or a determination may be made that a clinical trial presents unacceptable health risks;
- the methods and time required to determine whether the product candidate is effective may take longer than expected;
- fatalities or other adverse events arising during a clinical trial that may not be related to clinical trial treatments;
- the product candidate may appear to be no more effective than current therapies;
- the quality or stability of the product candidate may fail to conform to acceptable standards;

our inability to produce or obtain sufficient quantities of the product candidate to complete the trials;

our inability to reach agreement on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

our inability to obtain IRB approval to conduct a clinical trial at a prospective site;

our inability to obtain regulatory approval to conduct a clinical trial;

lack of adequate funding to continue the clinical trial, including the occurrence of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with the services of our CROs and other third parties;

Table of Contents

our inability to recruit and enroll individuals to participate in clinical trials for reasons including lack of patients or competition from other clinical trial programs for the same or similar indications; or

our inability to retain participants who have initiated a clinical trial but may be prone to withdraw due to side effects from the therapy, lack of efficacy or personal issues, or who are lost to further follow-up.

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

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

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

The timing and conduct of animal studies may be further constrained given that filoviruses are classified for use only in BSL-4 laboratories. There are limited laboratories and staff world-wide that can work with these live

Table of Contents

viruses and companies will be competing for the limited availability of this critical infrastructure to test their countermeasures. Furthermore, we anticipate limits in conforming to good laboratory practice (GLP) requirements given the requirement for BSL-4 containment.

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

We had an operating loss of $90.3 million for the year ended December 31, 2013 and incurred an operating loss of $29.7 million for the year ended December 31, 2012. As of December 31, 2013, our accumulated deficit was $543.2 million and substantially all of our revenues to date have been derived from research and development contracts with the DoD. We have not yet generated any material revenue from product sales and have incurred expenses related to research and development of our technology and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure and acquired in-process research and development resulting from two acquisitions. We anticipate that our expenses will increase substantially if and as we:

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

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

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

We will likely require additional capital from time to time in the future in order to continue the development of product candidates in our pipeline and to expand our product portfolio. The actual amount of funds that we may need will be determined by many factors, some of which are beyond our control. These factors include the success of our research and development efforts, the status of our preclinical and clinical

testing, costs and timing relating to securing regulatory approvals and obtaining new patent rights, regulatory changes, competitive and technological developments in the market and future commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

We would expect to seek additional financing from the sale and issuance of equity or equity-linked or debt securities, and we cannot predict that financing will be available when and as we need financing or that, if

Table of Contents

available, the financing terms will be commercially reasonable. If we are unable to obtain additional financing when and if we require it or on commercially reasonable terms, it would have a material adverse effect on our business and results of operations.

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. For example, through December 31, 2013, we sold an aggregate of approximately 3.4 million shares of our common stock in connection with our July 2013 at-the-market equity offering program. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs or to conduct clinical trials and to market our product candidates. Other than pre-clinical collaborations with academic/research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD and a product candidate for the treatment of influenza, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates other than that with the U.S. government. Such relationships may require us to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

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

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

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

Our RNA-based platforms, utilizing proprietary PMO-based technology, have not been incorporated into a therapeutic commercial product and are still at a relatively early stage of development. This technology is used in all of our product candidates, including eteplirsen. We are conducting toxicology, pharmacology, pharmacokinetics and other preclinical studies and, although we have conducted Phase I clinical trials for AVI-6003 (we are now pursuing development of AVI-7288, one of the two component oligomers in AVI-6003) and AVI-7100 and conducted a Phase IIb clinical trial in eteplirsen, additional preclinical studies may be required for these product candidates and before other product candidates enter human clinical trials. In addition, preclinical models to study participant toxicity and activity of compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease and there may be substantially different results in clinical trials from the results obtained in preclinical studies. Any failures or setbacks in utilizing our PMO-based technology, including adverse effects resulting from the use of this technology in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial position.

Table of Contents

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

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

The competition for qualified personnel in the biotechnology field and for qualified personnel with government contracting experience is intense, and our future success depends upon our ability to attract, retain and motivate such personnel. In order to develop and commercialize our products successfully, we will be required to retain key managerial, scientific and government contract compliance staff. In certain instances, we may also need to expand or replace 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.

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

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

For example, although there was no material error in our financial statements, in connection with our assessment of the effectiveness of internal control over financial reporting as of December 31, 2013, our management identified a material weakness in our internal control over financial reporting. A detailed description of this material weakness is provided in Item 9A, Controls and Procedures. We have adopted new procedures for the review of material contracts by our accounting staff to address this material weakness, however, we cannot assure you that material weaknesses in our internal control over financial reporting will not be identified in the future. Any failure to maintain or implement new or improved internal controls, or any difficulties that we may encounter in their maintenance or implementation, could result in additional material weaknesses or material misstatements in our financial statements and cause us to fail to meet our reporting obligations or prevent fraud, which could cause the trading price of our common stock to decline.

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

Table of Contents

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

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

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

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

- our patent rights might be challenged, invalidated, or circumvented, or otherwise might not provide any competitive advantage;
- as a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful; and
- jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop, and market competing products.

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

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

Additionally, the U.S. Supreme Court has issued decisions, the full impact of which are not yet known. For example, on March 20, 2012 in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc.*, the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears

Table of Contents

to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013 in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated natural products is uncertain and as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

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

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

Any of these events could substantially harm our potential earnings, financial condition, and operations. Prosensa Holding B.V. (Prosensa), which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. Prosensa has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office, and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of eteplirsen in the European Union would infringe on such patent. Both we and Prosensa have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other s briefs. The Opposition Division decision if maintained at the appeals level could have a substantial effect on our businesses and leaves open the possibility that Prosensa or other parties that have rights to such patent could assert that our drug eteplirsen infringes on such patent. The timing and outcome of appeal cannot be predicted or determined as of the date of this report. We are also aware of existing patent claims Prosensa is pursuing in the United States, and others that it is pursuing, in other jurisdictions, including Japan, that may provide the basis for Prosensa or other parties to assert that commercialization of eteplirsen would infringe on such claims.

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

Table of Contents

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

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

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

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

develop safer or more effective products;

implement more effective approaches to sales and marketing;

develop less costly products;

obtain regulatory approval more quickly;

have access to more manufacturing capacity;

develop products that are more convenient and easier to administer;

form more advantageous strategic alliances; or

establish superior intellectual property positions.

We may be subject to clinical trial claims and our insurance may not be adequate to cover damages.

We currently have no products that have been approved for commercial sale; however, the current and future use of our product candidates by us and our collaborators in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for any of our product candidates. However, we may not be able to

maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Table of Contents

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 cyber security incidents, could harm our ability to operate our business effectively.

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

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

In the ordinary course of business we may, and in some cases have, become involved in law-suits and other disputes such as securities claims, intellectual property challenges and employee matters. We may not prevail in claims made against us in such disputes. The outcome of such law suits and disputes is inherently uncertain. We do not believe that any of the current lawsuits or disputes faced by us is likely to have a material adverse effect on our financial condition, but the resolution of such lawsuits or disputes could have a material adverse effect on our results for that period. Also, lawsuits or claims brought against us in the future could have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Common Stock

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

The market prices for, and trading volumes of, securities of biotechnology companies, including our securities, have been historically volatile. For example, during 2013, our stock traded from a low of $12.89 per share to a high of $53.81 per share. As an additional example, we note that on November 12, 2013 our stock price decreased 64% on the same day that we made an announcement regarding eteplirsen and recent

Table of Contents

communications we had with the FDA. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including:

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

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

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

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

when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;

directors may only be removed for cause by the affirmative vote of majority of the voting power of all the then-outstanding shares of voting stock;

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

right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;

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

Table of Contents

prohibition on stockholder action by written consent;

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

the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and

a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

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

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

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Some of these fluctuations may be very pronounced such as in the case of the impact to our operating results as a result of our warrant offerings in January and August 2009 of which warrants for an aggregate of 0.8 million shares remain outstanding and exercisable as of December 31, 2013. Each of these warrants is classified as a derivative liability and 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 and comprehensive loss. For example, for the year ended December 31, 2013, the impact of the change in fair value of these warrants resulted in a $22.0 million loss in our consolidated statement of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option valuation model. Fluctuations in the assumptions and factors used in the Black-Scholes-Merton model can result in adjustments to the fair value of the warrants reflected on our balance sheet and, therefore, our statement of operations and comprehensive loss. Due to the classification of such warrants and other factors, results of operations are difficult to forecast, and period-to-period comparisons of our operating results may not be predictive of future performance. Additionally, our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Specifically, a change in the timing of activities performed in support of our U.S. government research contracts could either accelerate or defer anticipated revenue from period to period. Likewise, our research and development expenses may experience fluctuations as a result of the timing of activities performed in support of our U.S. government research contracts and the timing and magnitude of expenditures incurred in support of our DMD and other proprietary drug development programs. In one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

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

As of December 31, 2013, there were 37.8 million shares of common stock outstanding, outstanding awards to purchase 4.4 million shares of common stock under various incentive stock plans, and outstanding warrants to purchase up to 0.8 million shares of common stock. Additionally, as of December 31, 2013, there were 2.9 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity

Table of Contents

Incentive Plan and 0.3 million shares of common stock remain available for issuance under the Company s 2013 Employee Stock Purchase Plan. In addition, we may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock, perception that such issuances may occur, or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

A description of the facilities we own and/or occupy is included in the following table. We believe that our current facilities in Corvallis, Oregon and additional administrative and laboratory space in our Cambridge facility is suitable and will provide sufficient capacity to meet the projected needs of our business for the next 12 months. Except as noted below, all of our properties are currently being used in the operation of our business.

Location of Property	Square Footage	Lease Expiration Date	Purpose	Other Information
215 First Street, Suite 415, Cambridge, MA 02142	61,453	December 2020	Laboratory and office space	Corporate headquarters
215 First Street, Suite 7, Cambridge, MA 02142	7,087	February 2014	Office space	Temporary corporate headquarters
4575 SW Research Way, Suite 200, Corvallis, OR 97333	53,000	December 2020	Laboratory and office space	Primarily lab space
1749 SW Airport Avenue, Corvallis, OR 97333	36,150	N/A facility is owned; land lease expires February 2042	Acquired with intention of providing future expansion space for the manufacture of potential products and components	Approximately 25,000 square feet leased and the remaining space unoccupied*

* In November 2011, the tenant, Perpetua Power Source Technologies, Inc., or Perpetua, agreed to lease approximately 25,000 square feet of the building until March 2017. Perpetua may terminate the lease at the end of the 36th month upon 180 days prior written notice, together with delivery of a termination fee. Perpetua has the option to extend the lease for an additional year if notice is provided no less than 12 months prior to the expiration date. Perpetua also has a right of first refusal relating to the lease of the remaining space at the building and was granted an option to purchase the building during the term of the lease, provided there is no uncured default by Perpetua at the time of

exercise. If the purchase option is exercised, the price for the building is $2.0 million until February 2015, $2.1 from March 2015 until February 2016 and $2.2 million from March 2016 through the remainder of the initial lease term. If Perpetua exercises its extension option, the purchase price will be $2.3 million during the term of the extension.

Table of Contents

Item 3. Legal Proceedings.

As of December 31, 2013, the Company was 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 securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, in January 2014, a former consultant of the Company filed a complaint alleging breach of contract, among other claims, and seeking approximately $4 million in damages, plus certain additional fees and costs from the Company. In addition, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 (Corban v. Sarepta et al) and January 29, 2014 (Baradanian v. Sarepta et al). The plaintiffs are alleged purchasers of Company common stock who seek to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 24, 2013 and November 12, 2013. The complaints allege that the defendants violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seek damages in an unspecified amount. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

Item 4. Mine Safety Disclosures.

Not applicable.

Table of Contents

PART II

Item 5. Market for Registrant s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

As of January 2, 2014, our Common Stock is quoted on The NASDAQ Global Select Market under the symbol SRPT. Our Common Stock was quoted on The Nasdaq Global Market prior to January 2, 2014. The following table sets forth the high and low intraday sales prices as reported by The NASDAQ Global Market for each quarterly period in the two most recent years, including the effect of the reverse stock split:

	High	Low
Year Ended December 31, 2012		
First Quarter	$ 9.84	$ 4.20
Second Quarter	7.80	3.48
Third Quarter	16.44	3.30
Fourth Quarter	45.00	14.84
Year Ended December 31, 2013		
First Quarter	$ 37.70	$ 23.46
Second Quarter	42.20	28.90
Third Quarter	49.61	29.71
Fourth Quarter	55.61	12.12

Holders

As of February 24, 2014, we had 202 stockholders of record of our common stock.

Dividends

We have neither declared nor paid cash dividends on our common stock in 2013 or 2012. We currently expect to retain future earnings, if any, to finance the operation and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors.

Table of Contents

Performance Graph

The following graph compares the performance of our Common Stock for the periods indicated with the performance of the NASDAQ Composite Index and the Amex Biotech Index. This graph assumes an investment of $100 on December 31, 2008 in each of our common stock, the NASDAQ Composite Index and the Amex Biotech Index, and assumes reinvestment of dividends, if any. The stock price performance shown on the graph below is not necessarily indicative of future stock price performance. This graph is not soliciting material, is not deemed filed with the SEC and is not to be incorporated by reference into any of our filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing.

	SRPT	NASDAQ Composite Index	NASDAQ Biotech Index	NYSE Arca Biotech Index
End of Fiscal 2008	100.00	100.00	100.00	100.00
End of Fiscal 2009	221.77	145.34	115.96	145.58
End of Fiscal 2010	322.03	171.70	134.58	200.51
End of Fiscal 2011	113.16	170.34	150.85	168.74
End of Fiscal 2012	653.16	200.57	200.25	239.40
End of Fiscal 2013	515.70	281.14	332.45	361.02

Recent Sales of Unregistered Securities.

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers.

None.

Table of Contents

Item 6. Selected Financial Data.

The following selected financial data is derived from our audited financial statements and should be read in conjunction with, and is qualified in its entirety by, Item 7, Management s Discussion and Analysis of Financial Condition and Results of Operation, and Item 8, Financial Statements and Supplementary Data.

	Year Ended December 31,				
	2013	2012	2011	2010	2009
			(in thousands)		
Operations data:					
Revenues	$ 14,219	$ 37,329	$ 46,990	$ 29,420	$ 17,585
Research and development	72,909	52,402	66,862	35,972	24,396
General and administrative	31,594	14,630	16,055	14,382	8,696
Operating loss	(90,284)	(29,703)	(35,927)	(20,934)	(15,507)
Interest income (expense) and other, net	326	354	587	259	(454)
Gain (loss) on change in warrant valuation	(22,027)	(91,938)	33,022	(11,502)	(9,198)
Net loss	$ (111,985)	$ (121,287)	$ (2,318)	$ (32,177)	$ (25,159)
Net loss per share basic and diluted	$ (3.31)	$ (5.14)	$ (0.11)	$ (1.74)	$ (1.69)
Balance sheet data:					
Cash and cash equivalents	$ 256,965	$ 187,661	$ 39,904	$ 33,589	$ 48,446
Working capital	234,840	115,022	24,583	(8,019)	17,803
Total assets	291,569	204,993	54,368	45,976	60,027
Stockholders equity (deficit)	247,192	123,679	31,017	(2,817)	23,630

Table of Contents

Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve risks and uncertainties. Please review our legend titled Forward-Looking Information at the beginning of this Annual Report on Form 10-K which is incorporated herein by reference. Our actual results could differ materially from those discussed below. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled Risk Factors included elsewhere in this Annual Report on Form 10-K. Throughout this discussion, unless the context specifies or implies otherwise, the terms Sarepta , we , us and our refer to Sarepta Therapeutics, Inc. and its subsidiaries.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including our lead product candidate, eteplirsen. We are also focused on developing therapeutics for the treatment of infectious diseases, including our lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus. By building our infectious disease programs which are primarily funded and supported by the U.S. Department of Defense (DoD), and leveraging our highly-differentiated, proprietary technology platforms, we are seeking to further develop our research and development competencies and identify additional product candidates.

Our highly-differentiated RNA-based technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe unmet medical need. In 2012, we completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial. We are working with the FDA to initiate a pivotal clinical study in 2014 to determine the possibilities under expedited regulatory programs for eteplirsen.

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

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

Since our inception in 1980, we have incurred losses of $543.2 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on changes

Table of Contents

in warrant valuation partially offset by revenue generated from research contracts with and grants primarily from the DoD. As of December 31, 2013, we have completed all of our contracts with the DoD except for the July 2010 contract for the development of therapeutics against the Marburg virus. The period of performance for our August 2012 contract with the DoD concluded in the third quarter of 2013. In November 2012, we also entered into a consortium agreement with various parties that received an E.U. Health Innovation-1 2012 Collaborative research grant to support development of an exon 53-skipping therapeutic, based on our PMO chemistry, for which minimal revenues have been earned to date. We have not generated any material revenue from product sales to date, and there can be no assurance that revenues from product sales will be achieved. Moreover, even if we do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of December 31, 2013, we had $264.9 million of cash, cash equivalents and invested cash, comprised of $257.0 million of cash and cash equivalents and $7.9 million of restricted investments, which we believe, taking into consideration our outstanding warrants, is sufficient to fund our current operational plan for the next twelve months. Should our funding from the DoD cease or be delayed, we would likely curtail certain infectious disease research and development efforts unless additional funding was obtained. We are also likely to pursue additional cash resources through public or private financings, seeking additional government contracts, and by establishing collaborations or licensing our technology to other companies.

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

In the periods presented, nearly all of the revenue we generated was derived from research contracts with and grants from the U.S. government. As of December 31, 2013, we had completed all of our contracts with the DoD except for the Marburg portion of the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses.

The following table sets forth the revenue from each of our contracts with the U.S. and E.U. governments and other revenue for the years ended December 31, 2013, 2012 and 2011:

	Year Ended December 31,		
	2013	2012	2011
		(in thousands)	
July 2010 Agreement (*Ebola and Marburg IV*)	$ 9,064	$ 36,557	$ 42,875
June 2010 Agreement (*H1N1*)	427		3,490
May 2009 Agreement (*H1N1*)			516
August 2012 Agreement *(Intramuscular administration)*	2,791	673	
November 2012 SKIP-NMD Agreement (*DMD*)	1,263		
July 2013 Children s National Medical Center	674		
Other Agreements		99	109
Total	$ 14,219	$ 37,329	$ 46,990

The following is a description of each of our significant U.S. and E.U. government contracts and grants.

July 2010 Agreement (Ebola and Marburg)

On July 14, 2010, we were awarded a contract with the DoD Chemical and Biological Defense Program through the U.S. Army Space and Missile Defense Command for the advanced development of our hemorrhagic

Table of Contents

fever virus therapeutic candidates, AVI-6002 and AVI-6003, against the Ebola and Marburg viruses, respectively. The contract is a cost plus incentive fee (CPIF) type contract. Under a CPIF contract, the U.S. government pays the contractor s actual allowable costs incurred plus an incentive fee based on the contractor s performance against specified cost targets. In February 2012, we announced that we received permission from the FDA to proceed with a single oligomer from AVI-6003, AVI-7288, as the lead product candidate against the Marburg virus infection. On August 2, 2012, we received a stop-work-order related to the Ebola virus portion of this contract and, on October 2, 2012, the U.S. government terminated the Ebola portion of this contract for convenience of the U.S. government due to government funding constraints.

Our activities under the contract regarding AVI-7288 began in July 2010 and have included Phase I studies in healthy volunteers as well as preclinical studies. The remaining portion of the contract consists of the balance of the base segment and three optional segments of work for AVI-7288. At the end of each segment, the U.S. government assesses the progress of the development program and the availability of funding, among other things, to determine whether it will go forward and exercise the option for the next segment. Under the Federal Acquisition Regulation (FAR), the U.S. government has the unilateral right to exercise or not to exercise the options. If the U.S. government exercises an option, we have a duty to perform the optional segment (provided the U.S. government obligates sufficient funds to the contract). We cannot refuse to perform an optional segment of the work. The period of performance for the base segment of the contract ends on March 31, 2014. The U.S. government has no obligation to increase the funding of the contract and we have no obligation to incur costs in excess of the funded amount.

If the DoD exercises its options for all of the additional optional segments according to the scope of work in our contract, our contract activities would include clinical and licensure activities necessary to obtain FDA regulatory approval for our therapeutic candidate against the Marburg virus and would be scheduled to conclude in September 2016. Since DoD has not yet decided whether it will exercise these options, there is no funding obligated to the contract for their performance.

The rights of the U.S. government in inventions made in the performance of the contracts are set forth in FAR 52.227-11, Patent Rights-Ownership by the contractor, which is included in the DoD contract. In summary, FAR 52.227-11 gives contractors title to and the U.S. government a nonexclusive, nontransferable, irrevocable, paid up license to practice or have practiced for or on behalf of the United States any invention of contractor s made in the performance of work under the contract (i.e., a Subject Invention). In order to retain title to a Subject Invention, the contractor must disclose the invention to the U.S. government, formally elect to retain title, and file a patent application strictly in accordance with the detailed procedures and deadlines set forth in the clause. FAR 52.227-11 also includes a preference for domestic industry. In particular, contractors may not grant to any person the exclusive right to sell or use the invention in the United States unless such person agrees that any product embodying the invention or produced through the use of the invention will be manufactured substantially in the United States. In certain circumstances, this requirement may be waived by the U.S. government. Furthermore, under FAR 52.227-11, the U.S. government retains certain march in rights that permit the U.S. government to grant a license to the invention to a third party if: (1) the contractor has not taken effective steps to achieve practical application of the invention within a reasonable time; (2) such action is necessary to meet health and safety needs and/or requirements for public use that contractor is not meeting; and (3) contractor has not obtained the required agreement for manufacturing the invention in the United States from any exclusive licensee or a waiver of this requirement.

In addition to rights in inventions, the contract gives the U.S. government unlimited rights in technical data first produced in the performance of the contract and all data delivered under the contract. Unlimited rights means that the U.S. government has the rights to use, modify, reproduce, perform, display, release or disclose the data in whole or in part in any manner and for any purpose whatsoever and to have or authorize others to do so. Thus, there are no protections for technical data in which the U.S. government receives unlimited rights. However, under the clause, the contractor may withhold from delivery, data that embody trade secrets or are commercial or financial and confidential or privileged, to the extent that such data pertain to items,

Table of Contents

components, or processes developed at private expense, including minor modifications (i.e., limited rights data). If delivery of limited rights data is required and the contractor requires the U.S. government to keep this data confidential, the contractor must take certain steps prescribed in the regulations to protect this information.

Under FAR 52.249-6, Termination (Cost-Reimbursement) and the terms of this agreement, the U.S. government has the right to terminate the contract, in whole or in part, without prior notice, for its convenience or if the contractor defaults on its obligations under this agreement. The contractor has no right to terminate the contract for its convenience. In the event of a termination for convenience by the U.S. government, the contractor generally is entitled to recover its incurred cost plus a reasonable fee or profit on that incurred cost. It is not entitled to anticipatory fee or profit, i.e., the fee or profit it would have earned had the contract gone to completion.

For additional details regarding our remaining contract obligations with the U.S. government, see Note 6 Government Contracts of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K. For a description of the risks we face relating to our government contractual obligations see Risk Factors Risks Relating to Our Business .

June 2010 Agreement (H1N1/Influenza)

On June 4, 2010, we entered into a contract with the Defense Threat Reduction Agency (DTRA) to advance the development of AVI-7100 as a medical countermeasure against the pandemic H1N1 influenza virus in cooperation with the Joint Project Manager Transformational Medical Technologies program (or JPM-TMT), (renamed Medical Countermeasure Systems in 2013) of the U.S. Department of Defense, or DoD. The period of performance for this contract ended on June 3, 2011. We recognized $0.4 million associated with this agreement in 2013, which was the result of an indirect rate adjustment.

May 2009 Agreement (H1N1/Influenza)

In May 2009, we entered into a contract with DTRA to develop swine flu drugs using our proprietary PMO and PMO*plus*® antisense chemistry. In March 2010, the contract was amended to include testing against additional influenza strains. The key activities under this contract were completed in 2011.

August 2012 Agreement (Intramuscular administration)

On August 29, 2012, we were awarded a contract from the DoD s JPM-TMT program. The contract was for 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 period of performance for this contract concluded in the third quarter of 2013.

European Union SKIP-NMD Agreement (DMD)

In November 2012, we entered into a consortium agreement with various parties that received an E.U. Health Innovation-1 2012 Collaborative research grant to support development of an exon 53-skipping therapeutic, based on our PMO chemistry. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic and is expected to last approximately three years.

During the year ended December 31, 2013, we received $1.3 million in advance payments, which was fully recognized during the year ended December 31, 2013.

Table of Contents

July 2013 Children s National Medical Center (CNMC) Agreement

In July 2013, we entered into an agreement totaling $1.3 million to provide drug product to CNMC to conduct research related to the Company s DMD program. During the year ended December 31, 2013, the Company recognized $0.7 million as revenue under the agreement.

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 Government Contracts of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K. 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.

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

We defer recognition of non-refundable upfront fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee that is separate and independent of our performance under the other elements of the arrangement. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, then such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2013, we had deferred revenue of $3.3 million, which represents up-front fees which we will recognize as revenue as we satisfy the outstanding performance obligations.

Expenses

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

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

The amount and timing of future research and development expense will depend in part on our ability to obtain U.S. government awards to fund the advanced development of our infectious disease therapeutic candidates. Without such funding, we would likely significantly reduce our spending in these areas. Future research and development expenses may also increase as our internal projects, such as eteplirsen for DMD, enter later stage clinical development. Our research and development programs are currently in Phase IIb clinical trials or earlier 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.

Table of Contents

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, primarily consists of interest on our cash, cash equivalents, restricted investments, interest expense, and rental income. Our cash equivalents consist of money market investments. Interest expense includes interest paid on our mortgage loan related to the Corvallis property, the substantial portion of which we leased to a third party in November 2011. Rental income is from subleasing excess space in some of our facilities.

Gain (Loss) on Change in Warrant Valuation. Warrants issued in connection with our December 2007 and January and August 2009 financings are classified as liabilities as opposed to equity due to their settlement terms. These warrants are non-cash liabilities; we are not required to expend any cash to settle these liabilities. The fair market value of these warrants was recorded on the balance sheet at the date of issuance and the warrants are marked to market each financial reporting period, with changes in the fair value recorded as a gain or loss in our statement of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates related to the inputs used in the model and can result in significant swings in the fair market valuation primarily due to changes in our stock price. For more information, see Note 8 Warrants of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are based upon our consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The preparation of our consolidated financial statements in accordance with accounting principles generally accepted in the United States, or GAAP, requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, 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 consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

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

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

Revenue Recognition

We have historically generated revenue from our U.S. government research contracts and grants and other license arrangements.

Table of Contents

Government Research Contract Revenue. Substantially all of our revenue is generated from U.S. government research contracts and grants, which are generally cost plus contracts providing for reimbursed costs which include overhead and general and administrative costs and a target fee. We recognize revenue from U.S. government research contracts during the period in which the related expenses are incurred and present such revenues and related expenses gross in the consolidated financial statements.

License Arrangements. License arrangements may consist of non-refundable upfront license fees, data transfer fees, research reimbursement payments, exclusive licensed rights to patented or patent pending compounds, technology access fees, various performance or sales milestones and future product royalty payments. Some of these arrangements are multiple element arrangements. We defer recognition of non-refundable upfront fees if it has continuing performance obligations without which 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 and expertise related to the technology that is proprietary to us, or can only be performed by us, then such up-front fees are deferred and recognized over the period of continuing involvement.

Research and Development Expenses

All research and development expenses, including amounts funded through research and development collaborations, are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including salary and benefits; stock-based compensation expense; laboratory supplies and other direct expenses; contractual services, including clinical trial and pharmaceutical development costs; expenses associated with the supply investment in our drug candidates; and infrastructure costs, including facilities costs and depreciation.

When third-party service providers billing terms do not coincide with our period-end, we are required to make estimates of our obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of our drug candidates, incurred in a given accounting period and record accruals at the end of the period. We base our estimates on our knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable.

Table of Contents

Stock Compensation Expense

To determine stock-based compensation costs, we apply the provisions of Financial Accounting Standards Board (the FASB), Accounting Standards Codification (the ASC), Topic 718, Share-Based Payments. We use the Black-Scholes-Merton option pricing model for determining the estimated fair value for stock-based awards on the date of grant, which requires the use of subjective and complex assumptions to determine the fair value of stock-based awards, including the award s expected term and the price volatility of the underlying stock. We recognize the value of the portion of the awards that is ultimately expected to vest as expense over the requisite vesting periods on a straight-line basis for the entire award. Stock awards granted to employees are service-based and prior to December 31, 2010 typically vested 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, newly granted stock awards have a ten year term and typically vest over a four year period, with one fourth of the underlying shares vesting on the first anniversary of the grant and 1/48th of the underlying shares vesting monthly thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. The following table summarizes the weighted average assumptions used in determining the fair value of stock options granted:

	Year Ended December 31,		
	2013	2012	2011
Risk-free interest rate	0.7% - 1.7%	0.6% - 1.1%	0.9% - 2.4%
Expected dividend yield	%	%	%
Expected lives	4.8 - 5.0 years	4.8 - 5.3 years	5.2 - 8.9 years
Expected volatility	80.0% - 90.7%	79.7% - 108.6%	78.2% - 81.6%

The risk free interest rate is estimated using an average of U.S. Treasury bill interest rates over a historical period commensurate with the expected life of the option that correlates to the prevailing interest rates at the time of grant. The expected dividend yield is zero as we have not paid any dividends to date and do not expect to pay dividends in the future. The expected lives are estimated using expected and historical exercise behavior. For the year ended December 31, 2013, expected volatility was estimated using a blend of calculated volatility of the Company s common stock over a historical period and implied volatility in exchange-traded options with the Company s common stock. Prior to January 1, 2013, expected volatility was estimated using calculated volatility of the Company s common stock over a historical period commensurate with the expected term of the option. The amounts estimated according to the Black-Scholes-Merton option pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

The assumptions used in calculating the fair value of stock-based compensation expense represent management s best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and we use different assumptions, our stock-based compensation expense could be materially different in the future. See Note 3 Stock Compensation of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of stock-based compensation.

Warrant Liability

In December 2007 and January and August of 2009, we issued warrants to purchase an aggregate of 5.0 million shares of our common stock in connection with offerings of our common stock. These warrants are classified as a liability on our consolidated balance sheet due to their settlement terms. These warrants are non-cash liabilities; we are not required to expend any cash to settle these liabilities.

The fair value of the warrants is recorded on our consolidated balance sheet as a liability, and fair value is adjusted at each financial reporting period with the adjustment reflected in our consolidated statement of

Table of Contents

operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option pricing model, which requires the use of significant judgment and estimates related to the inputs used in the model. The following reflects the weighted-average assumptions for each of the periods indicated:

	Year Ended December 31,		
	2013	2012	2011
Risk-free interest rate	0.1%	0.2% - 0.3%	0.1% - 0.4%
Expected dividend yield	%	%	%
Expected lives	0.6 - 0.7 years	1.1 - 1.6 years	1.0 - 2.7 years
Expected volatility	95.51%	139.2% - 164.1%	71.8% - 75.6%

Fluctuations in the assumptions and factors used in the Black-Scholes-Merton model can result in adjustments to the fair value of the warrants reflected on our balance sheet and, therefore, our statement of operations and comprehensive loss. If, for example, the market value of our common stock or its volatility at December 31, 2013 were 10% higher or lower than what we used in the valuation of the warrants, our valuation of the warrants would have increased or decreased by $1.5 million due to the change in market price, and our valuation would have increased or decreased by $0.2 million due to the change in volatility, with the differences being reflected in our statement of operations and comprehensive los. See Note 8 Warrants of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of warrants.

Results of Operations for the years ended December 31, 2013, 2012 and 2011

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

Summary of Results for Fiscal Years 2013, 2012 and 2011

	Year Ended December 31,		
	2013	2012	2011
	(in thousands, except per share amounts)		
Operations data:			
Revenues	$ 14,219	$ 37,329	$ 46,990
Research and development	72,909	52,402	66,862
General and administrative	31,594	14,630	16,055
Operating loss	(90,284)	(29,703)	(35,927)
Interest income (expense) and other, net	326	354	587
Gain (Loss) on change in warrant valuation	(22,027)	(91,938)	33,022
Net loss	$ (111,985)	$ (121,287)	$ (2,318)
Net loss per share basic and diluted	$ (3.31)	$ (5.14)	$ (0.11)

Revenue

Revenue for 2013 decreased by $23.1 million, or 62%, compared to 2012. The decrease was due to a decrease of $27.5 million in revenue associated with July 2010 Ebola and Marburg agreement. The Ebola portion of the contract was terminated for convenience by the U.S. government due to lack of funding in the third quarter of 2012. Accordingly, there was no such revenue in 2013. These decreases in 2013 revenues were partially offset by revenue from the IM contract of $2.8 million in 2013 as compared to $0.7 million in 2012. Additionally, there were revenues from the November 2012 E.U. Skip and July 2013 CNMC agreements of $1.3 million and $0.7 million, respectively, which did not have any revenue associated with them in 2012.

Revenue for 2012 decreased by $9.7 million, or 21%, compared to 2011. The decrease was due to $4.0 million less revenue from the May 2009 and June 2010 H1N1 agreements which ended in 2011 and $6.3 million

Table of Contents

less revenue from the 2010 Ebola and Marburg contract. Of the $6.3 million from the 2010 Ebola and Marburg contract, $5.6 million was due to the Ebola portion of the contract being terminated for convenience by the U.S. government due to lack of funding in the third quarter of 2012. These decreases in 2012 revenue were partially offset by initial revenue from the IM contract of $0.7 million.

Research and Development Expenses

Research and development expenses for 2013 increased by $20.5 million, or 39%, compared to 2012. The increase was primarily due to a $31.3 million increase in our DMD program costs, as well as a $6.1 million increase in personnel related costs which includes a $2.7 million increase in stock-based compensation expense. The increase in DMD and personnel related costs were partially offset by a $17.3 million decrease in costs under the Ebola and Marburg contract with the DoD. This decrease was partially due to the August 2012 stop-work order and the subsequent termination for convenience in October 2012 on the Ebola portion of the contract as well as decreased activity on the Marburg portion of the contract.

Research and development expenses for 2012 decreased by $14.5 million, or 22%, compared to 2011. The decrease was due primarily to a $ 6.4 million decrease in non DMD proprietary research and a $4.1 million reduction in costs related to the Ebola portion of the July 2010 Ebola and Marburg contract which the U.S. government terminated due to a lack of funding in the third quarter of 2012, a $2.0 million reduction in costs resulting from the completion of the May 2009 and June 2010 H1N1 government contracts in 2011, a $1.0 million reduction due to the timing of various activities in both the Marburg portion of the July 2011 contract and the DMD program.

General and Administrative Expenses

General and administrative expenses for 2013 increased by $17.0 million, or 116%, compared to 2012. The increase in general and administrative expenses is primarily due to a $10.9 million increase in personnel costs including $5.3 million in stock-based compensation from additional headcount, $0.4 million of additional cost associated with facilities, $3.9 million of additional professional services and $1.8 million of other costs.

General and administrative expenses for 2012 decreased by $1.4 million, or 9%, compared to 2011. The decrease was primarily due to a decrease of $1.2 million in professional consulting services and $0.7 million in severance costs. These decreases were partially offset by $0.3 million of higher personnel costs due to filling vacant senior level positions.

Interest Income (Expense) and Other, Net

Interest income (expense) and other, net, for 2013 remained consistent compared to 2012 and 2011.

Gain (Loss) on Change in Warrant Valuation

The change in fair value of our warrant liability for 2013 compared to 2012 and 2011 was primarily attributable to the change in our stock price and warrant exercises. See Note 8 to the Notes of the Consolidated Financial Statements included elsewhere in this Annual Report on Form 10-K.

Net Loss

The decrease in our net loss of $9.3 million for 2013 compared to 2012 was primarily attributable to $69.9 million decrease in other income (loss), which was primarily due to the loss on change in our warrant valuation, which was partially offset by higher operating expenses and lower revenue in 2013 as compared to 2012.

Table of Contents

The increase in our net loss of $119.0 million for 2012 compared to 2011 was primarily attributable to a $125.0 million increase in other income (loss) resulting from an increase in the fair value of warrants accounted for as liabilities partially offset by a $6.2 million decrease in our operating loss.

Liquidity and Capital Resources

At December 31, 2013, our cash and cash equivalents were $257.0 million, compared to $187.7 million at December 31, 2012. Our cash and cash equivalents of $257.0 million does not include the $7.9 million in restricted investments, as those amounts secure letters of credit relating to certain lease and manufacturing agreements and are not currently available for general corporate use. The significant increase is due primarily to public offerings of our common stock and the exercise of outstanding warrants for the purchase of our common stock. Based on the factors described below, we believe that our currently available cash and cash equivalents is sufficient to finance our operations for the next 12 months.

Our principal sources of liquidity are revenue from our U.S. government research contracts and grants and equity transactions. Our principal uses of cash are research and development expenses, general and administrative expenses and other working capital requirements.

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 as was done regarding the Ebola portion of the July 2010 Ebola and Marburg contract in 2012. If U.S. government funding is not received or is delayed, we would likely curtail certain of our infectious disease research and development efforts unless additional funding was obtained. Currently, we do not generate any revenue from the commercial sale of our pharmaceutical product candidates.

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

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

Historical Trends

The following table sets forth sources and uses of funds activity for the period shown:

	Year Ended December 31,		
	2013	2012	2011
		(in thousands)	
Cash provided by (used in):			
Operating activities	$ (64,695)	$ (29,694)	$ (23,679)
Investing activities	(11,672)	(1,145)	(2,305)
Financing activities	145,671	178,596	32,299
Increase in cash and equivalents	$ 69,304	$ 147,757	$ 6,315

Table of Contents

Operating Activities.

The increase in the amount of cash used in operating activities of $35.0 million for 2013 compared to 2012 was primarily due to an increase in operating loss of $60.6 million driven by lower government contract revenue and higher research and development costs and higher general and administrative costs, partially offset by non-cash adjustments to net income in 2013 as compared to 2012 as well as changes in operating assets and liabilities.

The increase in the amount of cash used in operating activities of $6.0 million for 2012 as compared to 2011 was primarily due to a $12.2 million increase in cash used in changes in operating assets and liabilities in 2012 as compared to 2011, partially offset by a $6.2 million decrease in operating loss in 2012 as compared to 2011.

Investing Activities.

The increase in the amount of cash used in investing activities of $11.7 million for 2013 compared to 2012 was primarily due to the purchase of $7.3 million of investments in February 2013 to secure two letters of credit issued in connection with certain manufacturing contracts and due to the purchase of a $0.6 million investment to secure a letter of credit for a security deposit relating to our Cambridge lease. Additionally, capital expenditures increased by $2.4 million, primarily the result of the relocation of our corporate headquarters.

The decrease in the amount of cash used in investing activities of $1.2 million for 2012 compared to 2011 was primarily due to a decrease of $1.1 million in cash used for fixed asset purchases in 2012 as compared 2011.

Financing Activities.

Cash inflows from financing activities in 2013 were primarily the result of proceeds of $125.1 million from the sale of approximately 3.4 million shares of common stock under the 2012 and 2013 ATM sales agreements. We also received $18.2 million in net proceeds from warrant exercises and $2.7 million from stock option exercises during 2013 for which we issued approximately 2.6 million shares of additional common stock. These cash inflows were partially offset by debt repayments of $0.1 million and shares withheld for taxes on issuance of restricted stock units of $0.2 million.

Cash inflows from financing activities in 2012 were primarily the result of proceeds of $154.3 million from the sale of approximately 6.9 million shares of common stock through public offerings, as well as proceeds of $20.6 million from the issuance of approximately 1.7 million shares from warrant exercises and $3.8 million from the exercise of stock options. These cash inflows were partially offset by debt repayments of $0.1 million.

Cash inflows from financing activities in 2011 were primarily the result of proceeds of $32.1 million from the sale of approximately 3.8 million shares through common stock offerings, as well as proceeds of $0.3 million from stock option and warrant exercises. These cash inflows were partially offset by debt repayments of $0.1 million.

As of December 31, 2013, we had warrants outstanding to purchase approximately 792,000 shares of our common stock at an average price of $10.05. These warrants expire during 2014 and if they are all exercised, we would receive proceeds of $8.0 million.

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.

Table of Contents

Contractual Payment Obligations

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 contractual obligations arising from these arrangements as of December 31, 2013:

	Payments Due by Period				
	Total	Less than 1 Year	1-3 Years	3-5 Years	More than 5 Years
			(in thousands)		
Long-term debt(1)	$ 2,250	$ 171	$ 342	$ 343	$ 1,394
Operating leases	27,924	3,535	7,702	8,065	8,622
Purchases Obligations(2)	169,718	55,641	85,557	28,520	
Totals(3)	$ 199,892	$ 59,347	$ 93,601	$ 36,928	$ 10,016

1 Long-term debt consists of scheduled principal and interest payments on such debt. Interest on our long-term debt bears interest at a rate of 4.75% and matures in February 2027.

2 Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding to us and that specify all significant terms. Purchase obligations relate primarily to our DMD development program.

3 Under our agreement with the University of Western Australia (UWA), described further in Note 11 in the Notes to the Consolidated Financial Statements included elsewhere in this Annual Report on Form 10-K, we may be required to make certain upfront and milestone-based payments of up to $7.1 million. These potential milestone payments are not included in the above amounts because there is no guarantee the milestones will ever be met. As of December 31, 2013, we have made upfront payments of $1.1 million. Upon the first commercial sale of eteplirsen, we have agreed to pay UWA $1.0 million in milestone fees. For each additional product developed (up to five products), we have agreed to pay UWA milestone fees of $150,000, $350,000 and $500,000 upon initiation of Phase II trials, Phase III trials and regulatory approval, respectively.

Recent Accounting Pronouncements

See Note 2 Summary of Significant Accounting Policies Recent Accounting Pronouncements of the financial statements included elsewhere in this Annual Report on Form 10-K.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

As of December 31, 2013, we had $264.9 million of cash, cash equivalents and invested cash, comprised of $257.0 million of cash and cash equivalents and $7.9 million of restricted investments. As of December 31, 2012, we had cash and cash equivalents of $187.7 million. 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. A 0.1% decline in interest rates, occurring January 1, 2014 and sustained throughout the period ended December 31, 2014, would be inconsequential. Future declines in interest rates, however, would reduce investment income, but are not likely to be a material source of revenue to our company in the foreseeable future.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 begins on page F-1 in Item 15 of Part IV of this Annual Report on Form 10-K and is incorporated into this item by reference.

Table of Contents

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

(a) Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Annual Report on Form 10-K, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rule 13a-15 and 15d-15 under the Exchange Act. Based on that review, the principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were not effective as of December 31, 2013, because of the material weakness in internal control over financial reporting discussed in Management s Annual Report on Internal Control over Financial Reporting. Disclosure controls and procedures ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act (1) is recorded, processed, summarized, and reported within the time periods specified in the SEC rules and forms, and (2) is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

We do not expect that our disclosure controls and procedures will prevent all error and all fraud. A control procedure, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control procedure are met. Because of the inherent limitations in all control procedures, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our company have been detected. These inherent limitations include the realities that judgments in decision making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. We considered these limitations during the development of our disclosure controls and procedures, and will continually reevaluate them to ensure they provide reasonable assurance that such controls and procedures are effective.

Notwithstanding the ineffectiveness of our disclosure controls and procedures, we believe the consolidated financial statements present fairly in all material respects, our financial position, results of operations, and cash flows as of December 31, 2013 and 2012, and for each of the years in the three year period ended December 31, 2013, and the information included in the cumulative from inception presentations for the period of July 22, 1980 (inception) to December 31, 2013.

(b) Management s Annual Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting for our company, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act.

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on our financial statements.

Table of Contents

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

A material weakness is a deficiency or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company s annual or interim financial statements will not be prevented or detected on a timely basis.

Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2013. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in its *Internal Control Integrated Framework (1992)*. Based on this assessment, management identified a material weakness in the Company s internal control over financial reporting as follows: The Company did not design and implement controls to adequately review and consider the recognition and measurement of new significant research and development contracts.

This material weakness resulted in a reasonable possibility that a material misstatement of the Company s annual or interim consolidated financial statements related to research and development expenses and other current assets would not be prevented or detected. As a result of the aforementioned material weakness, management has concluded that, as of December 31, 2013, our internal control over financial reporting was not effective based on the criteria in the COSO *Internal Control Integrated Framework (1992)*.

KPMG LLP, an independent registered public accounting firm, has audited the consolidated financial statements included in this Annual Report on Form 10-K and, as part of its audit, has issued an adverse audit report on the effectiveness of our internal control over financial reporting as of December 31, 2013 which is included in this Form 10-K.

(c) Changes in Internal Control over Financial Reporting

Other than the identification of the material weakness described above, there have not been any changes in our internal control over financial reporting as defined in Rules 13a 15(f) and 15d 15(f) under the Exchange Act during the three months ended December 31, 2013 that our certifying officers concluded materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

In order to address the material weakness in internal control over financial reporting that was identified, management has designed and implemented procedures and controls to review and consider the recognition and measurement of all new significant research and development contracts. Such contracts will be reviewed by our accounting personnel with the requisite accounting knowledge, skills, and experience deemed necessary to perform such a review.

Table of Contents

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

Sarepta Therapeutics, Inc.:

We have audited Sarepta Therapeutics, Inc. s and subsidiaries (a development stage company) internal control over financial reporting as of December 31, 2013, based on criteria established in *Internal Control - Integrated Framework (1992)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Sarepta Therapeutics, Inc. s management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management s Annual Report on Internal Control over Financial Reporting (Item 9A(b)). Our responsibility is to express an opinion on the Company s internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company s internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company s internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company s assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company s annual or interim financial statements will not be prevented or detected on a timely basis. A material weakness related to the Company s design and implementation of controls to adequately review and consider the recognition and measurement of new significant research and development contracts has been identified and included in management s assessment.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries (a development stage company) as of December 31, 2013 and 2012, and the related consolidated statements of operations and comprehensive loss, stockholders equity (deficit), and cash flows for each of the years in the three-year period ended December 31, 2013 and the information included in the cumulative from inception presentations for the period January 1, 2002 to December 31, 2013 (not separately presented). This material weakness was considered in determining the nature, timing, and extent of audit tests applied in our audit of the 2013 consolidated financial statements, and this report does not affect our report dated March 3, 2014, which expressed an unqualified opinion on those consolidated financial statements.

Table of Contents

In our opinion, because of the effect of the aforementioned material weakness on the achievement of the objectives of the control criteria, Sarepta Therapeutics, Inc. has not maintained effective internal control over financial reporting as of December 31, 2013, based on criteria established in *Internal Control Integrated Framework (1992)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

/s/ KPMG LLP

Cambridge, Massachusetts

March 3, 2014

Item 9B. Other Information.

None.

Table of Contents

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information regarding our directors and executive officers required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2014 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2014 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2014 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2014 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2014 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Table of Contents

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) The following documents are filed as part of this Annual Report on Form 10-K:

(1) Financial Statements

The following consolidated financial statements of the Company and the Report of KPMG LLP, Independent Registered Public Accounting Firm, are included in Part IV of this Annual Report on Form 10-K on the pages indicated:

Report of KPMG LLP, Independent Registered Public Accounting Firm	F-1
Report of Arthur Andersen, Independent Public Accountants	F-2
Consolidated Balance Sheets	F-3
Consolidated Statements of Operations and Comprehensive Loss	F-4
Consolidated Statements of Stockholders Equity (Deficit)	F-5
Consolidated Statements of Cash Flows	F-6
Notes to Consolidated Financial Statements	F-7

(2) Financial Statement Schedules

All schedules are omitted because they are not applicable or the required information is shown in the consolidated financial statements or the notes thereto.

(3) Exhibits

The exhibits required by Item 601 of Regulation S-K are listed in paragraph (b) below.

(b) Exhibits.

The following exhibits are filed herewith or are incorporated by reference to exhibits previously filed with the SEC:

Exhibit		Incorporated by Reference to Filings Indicated				
Number	Description	Form	File No.	Exhibit	Filing Date	Provided Herewith
2.1	Agreement and Plan of Merger dated June 6, 2013 between Sarepta Therapeutics, Inc., a Delaware corporation, and Sarepta Therapeutics, Inc., an Oregon corporation.	8-K12B	001-14895	2.1	6/6/13	
3.1	Amended and Restated Certificate of Incorporation.	8-K12B	001-14895	3.1	6/6/13	
3.2	Bylaws.	8-K12B	001-14895	3.2	6/6/13	
4.1	Form of Specimen Certificate for Common Stock.	10-Q	001-14895	4.1	8/8/13	
4.2	Form of Common Stock Purchase Warrant, issued on January 30, 2009.	8-K	001-14895	4.4	1/30/09	
4.3	Form of Common Stock Purchase Warrant, issued on August 25, 2009.	8-K	001-14895	4.1	8/24/09	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.1	Employment Agreement with Patrick Iversen, Ph.D., dated July 14, 1997.	10KSB	000-22613	10.12	3/30/98	
10.2	Amendment to Employment Agreement with Patrick Iversen, Ph.D., dated December 28, 2008.	10-K	001-14895	10.5	3/15/11	
10.3	Amendment No. 2 to Employment Agreement with Patrick Iversen, Ph.D., dated January 18, 2010.	10-K	001-14895	10.6	3/15/11	
10.4	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between Sarepta Therapeutics, Inc. and Christopher Garabedian.	10-Q	001-14895	10.2	5/9/13	
10.5	Executive Employment Agreement dated January 10, 2011 by and between AVI BioPharma, Inc. and Effie Toshav.	10-Q	001-14895	10.1	5/10/11	
10.6	Executive Employment Agreement dated March 29, 2011 by and between AVI BioPharma, Inc. and Peter S. Linsley, Ph.D.	10-Q	001-14895	10.4	5/10/11	
10.7	Executive Employment Agreement dated June 13, 2011 by and between AVI BioPharma, Inc. and Edward Kaye, M.D.	10-Q	001-14895	10.4	8/8/11	
10.8	Stand Alone Stock Option Grant between AVI BioPharma, Inc. and Effie Toshav dated January 10, 2011.	10-Q	001-14895	10.2	5/10/11	
10.9	Stand Alone Stock Option Grant between the Registrant and Peter Linsley dated May 16, 2011.	S-8	333-175031	4.8	6/20/11	
10.10	Stand Alone Stock Option Grant between the Registrant and Edward Kaye dated June 20, 2011.	S-8	333-175031	4.9	6/20/11	
10.11	AVI BioPharma, Inc. 2002 Equity Incentive Plan.	Schedule 14A	001-14895	Appendix A	4/11/02	
10.12	Amended and Restated Sarepta Therapeutics, Inc. 2011 Equity Incentive Plan.	8-K12B	001-14895	10.1	6/6/13	
10.13	Form of Stock Option Award Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-Q	001-14895	10.5	8/8/13	
10.14	Form of Notice of Grant of Restricted Stock under the Amended and Restated 2011 Equity Incentive Plan.	10-Q	001-14895	10.4	8/8/13	
10.15	AVI BioPharma, Inc. Non-Employee Director Compensation Policy.	8-K	001-14895	10.85	10/1/10	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.16	Form of Indemnification Agreement.	8-K	001-14895	10.86	10/8/10	
10.17	Form of Restricted Stock Unit Award Agreement under 2011 Equity Incentive Plan.	8-K	001-14895	10.1	4/25/12	
10.18	Form of Stock Appreciate Right Award Agreement under the 2011 Equity Incentive Plan.	10-Q	001-14895	10.2	11/7/12	
10.19	Form of Senior Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.19	3/15/13	
10.20	Form of Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.20	3/15/13	
10.21	2013 Employee Stock Purchase Plan.	8-K12B	001-14895	10.2	6/6/13	
10.22	Executive Employment Agreement with Jayant Aphale, Ph.D.	10-Q	001-14895	10.1	8/8/13	
10.23	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.	10-Q	001-14895	10.3	5/9/13	
10.24	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and Sandesh Mahatme.					X
10.25	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and David Tyronne Howton.					X
10.26	Executive Inducement Stock Option Award Agreement between Arthur Krieg and Sarepta Therapeutics, Inc.					X
10.27	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan.					X
10.28	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan.					X
10.29*	Collaboration and License Agreement between Isis Pharmaceuticals and Ercole Biotech, Inc. dated May 16, 2003.	10-K	001-14895	10.78	3/16/10	
10.30*	Amended and Restated Exclusive License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc. and Sarepta International CV, dated April 10, 2013.	10-Q	001-14895	10.1	5/9/13	
10.31	Agreement between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency dated May 5, 2009.	10-Q	001-14895	10.72	8/10/09	
10.32	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA1-07-C-0010), effective May 29, 2009.	10-Q	001-14895	10.74	8/10/09	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated Form	File No.	Exhibit	Filing Date	Provided Herewith
10.33	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA 1-07-C0010), effective September 30, 2009.	10-Q	001-14895	10.77	11/9/09	
10.34*	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no HDTRA 1-09-C-0046), effective March 25, 2010.	10-Q	001-14895	10.81	5/10/10	
10.35*	Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. dated June 4, 2010.	10-Q	001-14895	10.84	8/9/10	
10.36*	Modification No. PZ0001 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective March 3, 2011.	10-Q	001-14895	10.3	5/10/11	
10.37*	Modification No. P00005 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective April 13, 2011.	10-Q	001-14895	10.1	8/8/11	
10.38*	Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. dated July 14, 2010.	10-Q	001-14895	10.86	11/9/10	
10.39*	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.	10-Q	001-14895	10.1	11/7/12	
10.40*	Modification No. P00005 to Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. effective August 15, 2011.	10-Q/A	001-14895	10.3	2/15/12	
10.41*	Sponsored Research Agreement between AVI BioPharma, Inc. and Charley s Fund, Inc., effective October 12, 2007.	10-K	001-14895	10.58	3/17/08	
10.42*	First Amendment to Sponsored Research Agreement between AVI BioPharma, Inc. and Charley s Fund, Inc. dated June 2, 2009.	10-Q	001-14895	10.75	8/10/09	
10.43	Commercial Lease between Research Way Investments, Landlord, and Antivirals, Inc., Tenant, effective June 15, 1992.	SB-2	333-20513	10.9	1/28/97	
10.44	Lease Extension and Modification Agreement dated September 1, 1996, by and between Research Way Investments and Antivirals, Inc.	10-K	001-14895	10.53	3/15/11	
10.45	Second Lease Extension and Modification Agreement dated January 24, 2006 by and between Research Way Investments and AVI BioPharma, Inc.	10-Q	001-14895	10.55	8/9/06	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.46	Real Property Purchase Agreement by and between WKL Investments Airport, LLC and AVI BioPharma, Inc., dated March 1, 2007, as amended.	10-Q	001-14895	10.61	8/9/07	
10.47	Lease Agreement between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc., dated November 23, 2011.	10-K	001-14895	10.42	3/13/12	
10.48	First Amendment to Lease Agreement dated December 22, 2011 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.43	3/13/12	
10.49	Second Amendment to Lease Agreement dated January 20, 2012 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.44	3/13/12	
10.50	Lease dated July 27, 2009 by and between BMR-3450 Monte Villa Parkway, LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.76	11/9/09	
10.51	First Amendment to Lease dated August 30, 2011 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.4	11/8/11	
10.52	Second Amendment to Lease dated January 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-K	001-14895	10.47	3/13/12	
10.53	Third Amendment to Lease dated May 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.2	8/7/12	
10.54	Lease dated October 20, 2010, by and between S/I North Creek VII LLC and AVI BioPharma, Inc.	10-K	001-14895	10.57	3/15/11	
10.55	Lease Agreement dated June 25, 2013 by and between Sarepta Therapeutics, Inc. and ARE-MA Region No. 38, LLC.	8-K	001-14895	10.1	7/1/13	
21.1	Subsidiaries of the Registrant.					X
23.1	Consent of Independent Registered Public Accounting Firm.					X
24.1	Power of Attorney (contained on signature page).					X
31.1	Certification of the Company s President and Chief Executive Officer, Christopher Garabedian, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company s Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X

Table of Contents

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

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

Table of Contents

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

Dated: March 3, 2014

SAREPTA THERAPEUTICS, INC.

By: /s/ Christopher Garabedian
Christopher Garabedian

President and Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christopher Garabedian and Sandesh Mahatme, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file, any and all documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their and his or her substitute or substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities indicated on March 3, 2014:

Signature	Title
/s/ Christopher Garabedian Christopher Garabedian	President, Chief Executive Officer and Director (Principal Executive Officer)
/s/ Sandesh Mahatme Sandesh Mahatme	Senior Vice President, Chief Financial Officer (Principal Financial and Accounting Officer)
/s/ William Goolsbee William Goolsbee	Chairman of the Board
/s/ M. Kathleen Behrens M. Kathleen Behrens, Ph.D.	Director
/s/ Anthony Chase Anthony Chase	Director
/s/ John C. Hodgman John C. Hodgman	Director

/s/ Gil Price	Director
Gil Price, M.D.	
/s/ Hans Wigzell	Director
Hans Wigzell, M.D., Ph.D.	

Table of Contents

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

Sarepta Therapeutics, Inc.:

We have audited the accompanying consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries (a development stage company) as of December 31, 2013 and 2012, and the related consolidated statements of operations and comprehensive loss, stockholders equity (deficit), and cash flows for each of the years in the three-year period ended December 31, 2013 and the information included in the cumulative from inception presentations for the period January 1, 2002 to December 31, 2013 (not separately presented). These consolidated financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. The financial statements of Sarepta Therapeutics, Inc. for the period July 22, 1980 (inception) to December 31, 2001 were audited by other auditors who have ceased operations. Those auditors expressed an unqualified opinion on those financial statements in their report dated February 21, 2002.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Sarepta Therapeutics, Inc. and subsidiaries (a development stage company) as of December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2013 and the information included in the cumulative from inception presentations for the period January 1, 2002 to December 31, 2013 (not separately presented), in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Sarepta Therapeutics, Inc. s internal control over financial reporting as of December 31, 2013, based on criteria established in *Internal Control Integrated Framework (1992)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated March 3, 2014 expressed an adverse opinion on the effectiveness of the Company s internal control over financial reporting.

/s/ KPMG LLP

Cambridge, Massachusetts

March 3, 2014

Table of Contents

THIS REPORT IS A CONFORMED COPY OF THE REPORT PREVIOUSLY ISSUED BY ARTHUR ANDERSEN LLP AND HAS NOT BEEN REISSUED BY THAT FIRM.

Report of Arthur Andersen, Independent Public Accountants

Report of Independent Public Accountants

To the Board of Directors and Shareholders of AVI BioPharma, Inc.

We have audited the accompanying balance sheet of AVI BioPharma, Inc. (an Oregon corporation in the development stage) as of December 31, 2001, and the related statements of operations, shareholders equity and cash flows for each of the two years in the period ended December 31, 2001 and for the period from inception (July 22, 1980) to December 31, 2001. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with auditing standards generally accepted in the United States. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of AVI BioPharma, Inc. as of December 31, 2001, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2001 and for the period from inception (July 22, 1980) to December 31, 2001, in conformity with accounting principles generally accepted in the United States.

/s/ Arthur Andersen LLP

Portland, Oregon

February 21, 2002

Table of Contents

Sarepta Therapeutics, Inc.

(A Development Stage Company)

Consolidated Balance Sheets

(in thousands, except share data)

	December 31, 2013	December 31, 2012
Assets		
Current Assets:		
Cash and cash equivalents	$ 256,965	$ 187,661
Accounts receivable	3,530	4,713
Restricted investments	7,250	
Other current assets	3,061	1,534
Total Current Assets	270,806	193,908
Restricted investments	647	
Property and equipment, net of accumulated depreciation and amortization of $17,328 and $16,708	15,049	3,397
Patent costs, net of accumulated amortization of $1,622 and $2,626	5,042	4,913
Other assets	25	2,775
Total Assets	$ 291,569	$ 204,993
Liabilities and Stockholders Equity		
Current Liabilities:		
Accounts payable	$ 17,634	$ 7,532
Accrued employee compensation	5,047	2,741
Current portion of long-term debt	92	89
Warrant liability	9,006	65,193
Deferred revenue	3,299	3,304
Other current liabilities	888	27
Total Current Liabilities	35,966	78,886
Long-term debt	1,576	1,668
Deferred rent and other long-term liabilities	6,835	760
Total Liabilities	44,377	81,314
Commitments and contingencies		
Stockholders Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding		
Common stock, $.0001 par value, 50,000,000 shares authorized; 37,751,920 and 31,703,817 issued and outstanding at December 31, 2013 and 2012, respectively	4	3
Additional paid-in capital	790,424	554,927
Deficit accumulated during the development stage	(543,236)	(431,251)
Total Stockholders Equity	247,192	123,679
Total Liabilities and Stockholders Equity	$ 291,569	$ 204,993

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

(A Development Stage Company)

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except per share data)

	Year ended December 31,			July 22, 1980 (Inception) through December 31,
	2013	2012	2011	2013
Revenues from license fees, grants and research contracts	$ 14,219	$ 37,329	$ 46,990	$ 187,767
Operating expenses:				
Research and development	72,909	52,402	66,862	458,577
General and administrative	31,594	14,630	16,055	150,681
Acquired in-process research and development				29,461
Operating loss	(90,284)	(29,703)	(35,927)	(450,952)
Other income (loss):				
Interest income (expense) and other, net	326	354	587	9,849
Gain (loss) on change in warrant valuation	(22,027)	(91,938)	33,022	(88,995)
Realized gain on sale of short-term securities available-for-sale				3,863
Write-down of short-term securities available-for-sale				(17,001)
Total other income (loss):	(21,701)	(91,584)	33,609	(92,284)
Net loss	$ (111,985)	$ (121,287)	$ (2,318)	$ (543,236)
Other comprehensive income (loss):				
Write-down of short-term securities available-for-sale				17,001
Realized gain on sale of short-term securities available-for-sale				(3,863)
Unrealized loss on short-term securities available-for-sale				(13,138)
Total other comprehensive income (loss)				
Comprehensive loss	$ (111,985)	$ (121,287)	$ (2,318)	$ (543,236)
Loss per share:				
Net loss per share basic and diluted	$ (3.31)	$ (5.14)	$ (0.11)	
Weighted average number of common shares outstanding for computing basic and diluted net loss per share (in thousands)	33,850	23,602	21,599	

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

(A Development Stage Company)

Consolidated Statements of Stockholders Equity (Deficit)

(in thousands)

	Partnership Units	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Deficit Accumulated During the Development Stage	Total Stockholders Equity (Deficit)
BALANCE AT JULY 22, 1980 (Inception)			**$**	**$**	**$**	**$**
Issuance of partnership units, warrants and common stock	602	1,379		33,734		33,734
Compensation expense related to issuance of warrants for common stock and partnership units				537		537
Exercise of warrants for partnership units and common stock	7	375		4,152		4,152
Exercise of options for common stock		467		6,221		6,221
Exercise of warrants for common stock		51		549		549
Issuance of common stock for ESPP		163		2,417		2,417
Issuance of common stock and warrants for cash and securities, net of offering costs		14,400	2	207,320		207,322
Issuance of common stock and warrants for the acquisition of business interests		1,324		25,559		25,559
Issuance of common stock and warrants to vendors		143		3,297		3,297
Stock-based compensation, net of cancellations of restricted stock		160		21,419		21,419
Conversion of debt into common stock and partnership units	2	2		88		88
Issuance of common stock in exchange for partnership units	(302)	272				
Withdrawal of partnership net assets upon conveyance of technology	(309)			(177)		(177)
Common stock subject to rescission, net		(11)		(289)		(289)
Net loss					(307,646)	(307,646)
BALANCE AT DECEMBER 31, 2010		**18,725**	**2**	**304,827**	**(307,646)**	**(2,817)**
Exercise of options for common stock		30		166		166
Exercise of warrants for common stock		35		759		759
Issuance of common stock for cash, net of offering costs		3,834		32,098		32,098
Stock-based compensation, net of cancellations of restricted stock				3,129		3,129
Net loss					(2,318)	(2,318)
BALANCE AT DECEMBER 31, 2011		**22,624**	**2**	**340,979**	**(309,964)**	**31,017**
Exercise of options for common stock		372		3,780		3,780
Exercise of warrants for common stock		1,770		52,742		52,742
Issuance of common stock for cash, net of offering costs		6,934	1	154,348		154,349
Stock-based compensation, net of cancellations of restricted stock		4		3,078		3,078
Net loss					(121,287)	(121,287)
BALANCE AT DECEMBER 31, 2012		**31,704**	**$ 3**	**$ 554,927**	**$ (431,251)**	**$ 123,679**
Exercise of options for common stock		241		2,725		2,725
Exercise of warrants for common stock		2,336		96,768		96,768
Restricted stock unit issuance		31				
Shares withheld for taxes		(7)		(226)		(226)
Restricted stock awards granted		6				
Issuance of common stock for cash, net of offering costs		3,441	1	125,103		125,104

Stock-based compensation, net of cancellations of restricted stock			11,127		11,127
Net loss				(111,985)	(111,985)
BALANCE AT DECEMBER 31, 2013	**37,752**	**$ 4**	**$ 790,424**	**$ (543,236)**	**$ 247,192**

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

(A Development Stage Company)

Consolidated Statements of Cash Flows

(in thousands)

	Year ended December 31,			For the Period July 22, 1980 (Inception) through December 31,
	2013	2012	2011	2013
Cash flows from operating activities:				
Net loss	$ (111,985)	$ (121,287)	$ (2,318)	$ (543,236)
Adjustments to reconcile net loss to net cash flows used in operating activities:				
Depreciation and amortization	1,277	1,525	1,300	23,247
Loss on abandonment of patents and disposal of property and equipment	590	357	190	3,226
Realized gain on sale of short-term securities available-for-sale				(3,863)
Write-down of short-term securities available-for-sale				17,001
Impairment charge on real estate owned			109	1,445
Stock-based compensation	11,127	3,078	3,129	43,200
Acquired in-process research and development				29,461
Increase (decrease) on warrant valuation	22,027	91,938	(33,022)	88,995
Net increase (decrease) in accounts receivable, other current assets and other assets	2,801	(3,587)	(1,063)	(5,960)
Net increase (decrease) in accounts payable, accrued employee compensation, and other liabilities	9,468	(1,718)	7,996	21,881
Net cash used in operating activities	(64,695)	(29,694)	(23,679)	(324,603)
Cash flows from investing activities:				
Purchase of restricted investments	(7,897)			(7,897)
Purchase of property and equipment	(2,370)	(108)	(1,178)	(22,357)
Patent costs	(1,405)	(1,037)	(1,127)	(11,934)
Purchase of marketable securities				(112,993)
Sale of marketable securities				117,724
Acquisition costs				(2,389)
Net cash used in investing activities	(11,672)	(1,145)	(2,305)	(39,846)
Cash flows from financing activities:				
Proceeds from exercise of options and warrants, and the sale of common stock, warrants, and partnership units, net of offering costs	145,986	178,681	32,380	622,545
Repayments of long-term debt	(89)	(85)	(81)	(519)
Other financing activities, net	(226)			(612)
Net cash provided by financing activities	145,671	178,596	32,299	621,414
Increase in cash and cash equivalents	69,304	147,757	6,315	256,965
Cash and cash equivalents:				

Beginning of period	187,661	39,904	33,589	
End of period	$ 256,965	$ 187,661	$ 39,904	$ 256,965
Supplemental disclosure of cash flow information:				
Cash paid during the year for interest	$ 144	$ 86	$ 90	$ 719
Supplemental schedule of noncash investing activities and financing activities:				
Short-term securities available-for-sale received in connection with the private offering	$	$	$	$ 17,897
Issuance of common stock in satisfaction of warrants and other liabilities	$ 78,214	$ 32,191	$ 643	$ 111,048
Tenant improvements paid by landlord	$ 6,214	$	$	$ 6,214
Property and equipment included in accounts payable and other liabilities	$ 3,964	$	$	$ 3,964
Issuance of common stock for building purchase	$	$	$	$ 750
Assumption of long-term debt for building purchase	$	$	$	$ 2,200
Issuance of common stock to acquire assets	$	$	$	$ 8,075
Assumption of liabilities to acquire assets	$	$	$	$ 2,124

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. ORGANIZATION AND NATURE OF BUSINESS

Business

Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries (Sarepta or the Company) is a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying the Company s proprietary platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. The Company is focused on advancing the development of its Duchenne muscular dystrophy (DMD) drug candidates, including its lead product candidate, eteplirsen, for which the Company is currently conducting an ongoing open label extension study following completion of its initial Phase IIb clinical trials. The Company is also focused on developing therapeutics for the treatment of infectious diseases, including its lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus for which the Company has historically received significant financial support from U.S. government research contracts.

Since its inception in 1980, the Company has incurred losses of approximately $543.2 million, substantially all of which resulted from expenditures related to research and development and general and administrative charges partially offset by revenue generated from research contracts with and grants from the U.S. government. As of December 31, 2013, the Company has completed all of its contracts with the U.S. government except for the July 2010 agreement and the August 2012 agreement for the development of therapeutics against the Marburg virus. 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 is likely to continue to incur operating losses in the near term.

As of December 31, 2013, the Company had $264.9 million of cash, cash equivalents and invested cash, comprised of $257.0 million of cash and cash equivalents and $7.9 million of restricted investments, which the Company believes, taking into consideration outstanding warrants, is sufficient to fund its current operational plan for the next twelve months. Should the Company s funding from the U.S. government cease or be delayed, the Company would likely curtail certain of its infectious disease research and development efforts unless additional funding was obtained. The Company is also likely to pursue additional cash resources through public or private financings, seeking additional government contracts, and from establishing collaborations or licensing its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying consolidated financial statements reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All inter-company transactions between and among its consolidated subsidiaries have been eliminated. The consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others.

Estimates and Uncertainties

The preparation of consolidated 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

Table of Contents

contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reporting period. Actual results could differ from those estimates. Significant items subject to such estimates and assumptions include the valuation of stock-based awards and liability classified warrants, research and development expenses, and revenue recognition.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of 90 days or less from the date of purchase to be cash equivalents.

Accounts Receivable

Accounts receivable are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily 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 $2.4 million and $3.2 million of receivables from the U.S. government that were unbilled at December 31, 2013 and 2012, respectively.

Property and Equipment

Property and equipment are carried at cost, subject to review for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable. The cost of normal, recurring, or periodic repairs and maintenance activities related to property and equipment are expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the repair will result in future economic benefits.

The Company generally depreciates the cost of its property and equipment using the straight-line method over the estimated useful lives of the respective assets, which are summarized as follows:

Asset Category	Useful lives
Lab equipment	5 years
Office equipment	5 years
Leasehold improvements	Lesser of the useful life or the term of the respective lease
Building	30 years
Construction in Progress	Not depreciated until put into service

Amounts included in property and equipment are as follows:

	As of December 31,	
	2013	2012
	(in thousands)	
Lab equipment	$ 7,728	$ 6,890
Office equipment	1,432	1,301
Leasehold improvements	10,058	10,058
Building	1,856	1,856
Construction in progress	11,303	
Property and equipment, gross	32,377	20,105
Less accumulated depreciation	(17,328)	(16,708)
Property and equipment, net	$ 15,049	$ 3,397

At December 31, 2013, the Company recorded construction in progress related to the Cambridge headquarters lease totaling $11.3 million, of which $6.2 million was paid by the landlord as part of a tenant improvement allowance which is recorded in other long-term liabilities. These assets will be placed into service during the first quarter of fiscal 2014.

In 2009, the Company listed for sale the industrial property it owns in Corvallis, Oregon. In connection with this decision, the Company classified the property as Property held for sale and ceased depreciating the

Table of Contents

property. While the property was held for sale, the Company, with the assistance of independent appraisals, periodically estimated the fair market value less the costs to sell the property and in 2011 and 2010, recorded impairment charges of $0.1 million and $0.4 million, respectively. In November 2011, the Company leased approximately 70% of the building to a third party through March 31, 2017 at rates ranging from $14,500 per month to $15,500 per month. Under the terms of the agreement, the third party can terminate the lease in November 2014 upon proper notice and delivery of a termination fee. In addition, the third party has the option to purchase the building for prices ranging from $2.0 million to $2.2 million during the initial lease term. Upon entering into the lease agreement, the Company reclassified the $1.9 million carrying value of the building from Property held for sale to Property and equipment and began depreciating the building over 30 years which is the remaining term of the ground lease. Rent earned on the building is recorded as Interest income and other, net and was $0.2 million and $0.1 million in 2013 and 2012, respectively.

Depreciation expense was $0.8 million in 2013, $1.0 million in 2012, and $0.8 million in 2011.

Patent Costs

Patent costs consist primarily of external legal costs, filing fees incurred to file patent applications and renewal fees on proprietary technology developed or licensed by the Company. Patent costs associated with applying for a patent, being issued a patent and annual renewal fees are capitalized. Costs to defend a patent and costs to invalidate a competitor s patent or patent application are expensed as incurred. Patent costs are amortized on a straight-line basis over the shorter of the estimated economic lives or the initial term of the patents, generally 20 years. Patent amortization expense was $0.4 million, $0.6 million and $0.5 million for the years ended December 31, 2013, 2012 and 2011, respectively. The Company also expensed the remaining net book value of previously capitalized patents that were later abandoned of $0.5 million, $0.4 million and $0.2 million in 2013, 2012 and 2011, respectively. The Company expects to incur amortization expense of approximately $0.5 million per year over the next five years based on the unamortized patent costs as of December 31, 2013.

Revenue Recognition

Government Research Contract Revenue. Substantially all of the Company s revenue is generated from U.S. government research contracts and grants. See Note 6 Government Contracts. The Company s contracts with the U.S. government are cost plus contracts providing for reimbursed costs which include overhead and general and administrative costs and a target fee. The Company recognizes revenue from U.S. government research contracts during the period in which the related expenses are incurred and presents such revenues and related expenses gross in the consolidated financial statements.

License Arrangements. 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. The Company defers recognition of non-refundable upfront fees if it has continuing performance obligations without which 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 Company performance under the other elements of the arrangement. In addition, if the Company has continuing involvement through research and development services that are required because of its know-how and expertise related to the technology that is proprietary to the Company, or can only be performed by the Company, then such up-front fees are deferred and recognized over the period of continuing involvement.

Research and Development

Research and development costs are expensed as incurred.

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

Table of Contents

Direct research and development expenses associated with the Company s 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 the Company s clinical program include salaries, stock based compensation, and an allocation of the Company s facility costs. When third-party service providers billing terms do not coincide with the Company s period-end, the Company is required to make estimates of its obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of its drug candidates, incurred in a given accounting period and record accruals at the end of the period. The Company bases its estimates on its knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable.

Stock Compensation

The Company issues stock options, stock appreciation rights, restricted stock and restricted stock units to certain employees, officers and directors. The Company accounts for stock compensation using the fair value method, which results in the recognition of compensation expense over the vesting period of the awards. See Note 3 Stock Compensation for additional information.

Income Taxes

The Company follows the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. It is the intention of the Company to reinvest the earnings of its non-U.S. subsidiaries in those operations and not to repatriate the earnings to the United States. Accordingly, the Company does not provide for deferred taxes on the excess of the financial reporting over the tax basis in its investments in foreign subsidiaries as they are considered permanent in duration. To date, the Company has not had any earnings in its non-U.S. subsidiaries.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered and settled. A valuation allowance is recorded to reduce the net deferred tax asset to zero because it is more likely than not that the net deferred tax asset will not be realized. The Company recognizes the effect of income tax positions only if those positions are more likely than not of being sustained upon an examination.

Fair Value of Financial Instruments

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.

Table of Contents

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 December 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Restricted investments, current	$ 7,250	$ 7,250	$	$
Restricted investments, noncurrent	647	647		
Total assets	$ 7,897	$ 7,897	$	$

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

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

*See Note 8 Warrants for additional information related to the determination of fair value of the warrants.

As of December 31, 2012, there were no restricted investments held by the Company. The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable, and accounts payable approximate fair value because of the immediate or short-term maturity of these financial instruments and carrying amounts reported for long-term debt approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

Rent Expense

The Company s operating leases for its Cambridge, Massachusetts and Corvallis, Oregon facilities provide for scheduled annual rent increases throughout each lease s term. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full term of the leases, which expire in 2020 for the Cambridge, Massachusetts and Corvallis, Oregon facilities.

Commitments and Contingencies

The Company records liabilities for legal and other contingencies when information available to the Company indicates that it is probable that a liability has been incurred and the amount of loss can be reasonably estimated. Legal costs in connection with legal and other contingencies are expensed as costs are incurred.

Long-Lived Asset Impairment

Long-lived assets held and used by the Company and intangible assets with determinable lives are reviewed for impairment whenever events or circumstances indicate that the carrying amount of assets may not be

Table of Contents

recoverable. The Company evaluates recoverability of assets to be held and used by comparing the carrying amount of an asset to future net undiscounted cash flows to be generated by the asset. If such assets are considered to be impaired, the impairment to be recognized is measured as the amount by which the carrying amount of the assets exceeds the fair value of the assets. Such reviews assess the fair value of the assets based upon estimates of future cash flows that the assets are expected to generate.

The Company conducts periodic evaluations of the value of its patents. Pursuant to these evaluations, the Company recorded charges of $0.5 million, $0.4 million and $0.2 million in 2013, 2012 and 2011, respectively, for previously capitalized costs related to patents that were abandoned.

Recent Accounting Pronouncements

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

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

3. STOCK COMPENSATION

The Company previously sponsored a 2002 Equity Incentive Plan (the 2002 Plan) pursuant to which it issued options to purchase its common stock to the Company s employees, directors and service providers. In June 2011, the 2002 Plan was replaced by the 2011 Equity Incentive Plan (the 2011 Plan and, together with the 2002 Plan, the Plans) following approval by the Company s stockholders. There will be no further grants under the 2002 Plan. The 2011 Plan allows for the grant of stock options, stock appreciation rights, restricted stock awards, restricted stock units, performance shares and performance units. As of December 31, 2013, 2,879,000 shares of common stock remain available for future grant. In June 2013, the Company s stockholders approved the 2013 Employee Stock Purchase Plan (ESPP) with 250,000 shares of common stock available to be issued as of December 31, 2013.

Stock Options

Historically, stock options granted under the 2002 Plan prior to December 31, 2010 vested over a three year period, with one-third of the underlying shares vesting on each anniversary of grant, and have a ten year term, subject to the terms of the applicable plan under which they were granted. Beginning in January 2011, stock options granted under the 2002 Plan vest over a four year period, with one-fourth of the underlying shares vesting on the first anniversary of the grant and 1/48th of the underlying shares vesting monthly thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant.

Stock options granted under the 2011 Plan have a ten year term and vest over a four year period, with one-fourth of the underlying shares vesting on the first anniversary of the grant and 1/48th of the underlying shares vesting monthly thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant, subject to the terms of the applicable plan under which they were granted.

Table of Contents

The Company s stock option activity consisted of the following as of the dates indicated:

	For the Year Ended December 31,					
	2013		2012		2011	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Options outstanding at beginning of year	2,522,522	$ 11.76	2,417,659	$ 11.18	1,415,009	$ 12.84
Granted	2,283,719	34.18	1,269,470	12.92	1,595,375	9.18
Exercised	(241,056)	11.31	(371,353)	10.18	(25,291)	6.54
Canceled or expired	(374,818)	16.83	(793,254)	12.59	(567,434)	9.90
Options outstanding at end of year	4,190,367	$ 23.46	2,522,522	$ 11.76	2,417,659	$ 11.18
Exercisable at year end	1,051,329	$ 11.91	615,394	$ 12.71	742,211	$ 15.60
Vested at December 31, 2013 and expected to vest	3,467,069	$ 21.50				

	Aggregate Intrinsic Value	Weighted Average Remaining Contractual Life (Years)
Options outstanding at end of year	$ 18,544,313	8.58
Exercisable at end of year	9,856,124	7.04
Vested at December 31, 2013 and expected to vest	18,069,138	8.41

The weighted-average fair value per share of stock options granted during the 2013, 2012 and 2011 was $22.86, $9.54 and $6.12, respectively. During the same periods, the total intrinsic value of stock options exercised was $5.4 million, $5.0 million, and $0.1 million, respectively. The total grant date fair value of stock options vested for 2013, 2012 and 2011 was $4.9 million, $3.7 million and $2.8 million, respectively.

During 2013, 2012 and 2011, $2.7 million, $3.8 million and $0.2 million, respectively, was received upon the exercise of stock options.

Valuation Assumptions

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

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

	Year Ended December 31,		
	2013	2012	2011
Risk-free interest rate	0.7% - 1.7%	0.6% - 1.1%	0.9% - 2.4%
Expected dividend yield	0%	0%	0%
Expected lives	4.8 - 5.0 years	4.8 - 5.3 years	5.2 - 8.9 years
Expected volatility	80.0% - 90.7%	79.7% - 108.6%	78.2% - 81.6%

The risk-free interest rate is estimated using an average of treasury bill interest rates over a historical period commensurate with the expected term of the option that correlates to the prevailing interest rates at the time of grant. The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect

Table of Contents

to pay dividends in the future. The expected lives are estimated using expected and historical exercise behavior. For the year ended December 31, 2013, expected volatility was estimated using a blend of calculated volatility of the Company s common stock over a historical period and implied volatility in exchange-traded options of the Company s common stock. Prior to January 1, 2013, expected volatility was estimated using calculated volatility of the Company s common stock over a historical period commensurate with the expected term of the option. The amounts estimated according to the Black-Scholes-Merton option pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

The Company is required to estimate potential forfeiture of stock grants and adjust compensation cost recorded accordingly. The estimate of forfeitures is adjusted over the requisite service period to the extent that actual forfeitures differ, or are expected to differ, from such estimates. Changes in estimated forfeitures are recognized through a cumulative catch-up in the period of change and impact the amount of stock compensation expense to be recognized in future periods.

Restricted Stock Awards

In 2013, 2012 and 2011, the Company granted a total of 6,000, 4,998 and 5,000 shares of restricted stock, respectively to members of its board of directors. These shares vest on the first anniversary of the grant. During 2013, 2012 and 2011, the Company recognized compensation expense related to these shares of $149 thousand, $38 thousand and $22 thousand, respectively.

The following table sets forth restricted stock activity for the years shown:

	For the Year Ended December 31,					
	2013		2012		2011	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Restricted Stock Awards outstanding at beginning of year	4,998	$ 10.08	5,000	$ 8.46	3,333	$ 7.80
Granted	6,000	34.92	4,998	10.08	5,000	8.46
Vested	(4,998)	10.08	(5,000)	8.46	(3,333)	7.80
Forfeited or canceled						
Restricted Stock Awards outstanding at end of year	6,000	$ 34.92	4,998	$ 10.08	5,000	$ 8.46

The weighted-average grant-date fair value of restricted stock awards is the market price of the Company s common stock on the date of grant which is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The total grant-date fair values of restricted stock awards that vested during 2013, 2012 and 2011 were approximately $50 thousand, $42 thousand and $26 thousand, respectively.

Table of Contents

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 2011 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 Company did not grant any RSUs in 2013 or 2011. 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 which is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The following table sets forth restricted stock unit activity for the period shown:

	For the Year Ended December 31,			
	2013		2012	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Restricted Stock Units outstanding at beginning of year	38,260	$ 6.32		$
Granted			39,877	6.28
Vested	(31,379)	6.52		
Forfeited or canceled	(374)	5.40	(1,617)	5.40
Restricted Stock Units outstanding at end of year	6,507	$ 5.40	38,260	$ 6.32

The weighted-average grant-date fair value of restricted stock units is the market price of the Company s common stock on the date of grant which is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The total grant-date fair value of restricted stock units that vested during 2013 was approximately $0.2 million.

Stock Appreciation Rights

The Company issues Stock Appreciation Rights (SARs) to employees on the same terms as options granted to employees. The grant date fair value of the SARs is determined using the same valuation assumptions as for stock options described above. Stock-based compensation expense is recognized on a straight-line basis over the vesting period of the SARs.

In August 2012, 70,000 SARs were granted to the Company s President and CEO and have an exercise price of $10.08 per share. In November 2012, 100,000 SARs were granted to the Company s Senior Vice-President and CFO and have an exercise price of $23.85 per share. The SARs are classified as equity as the agreements require settlement in shares of stock. The following table sets forth stock appreciation rights activity for the period shown:

	For the Year Ended December 31,			
	2013		2012	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Stock Appreciation Rights outstanding at beginning of year	170,000	$ 18.18		$
Granted			170,000	18.18
Stock Appreciation Rights outstanding at end of year	170,000	$ 18.18	170,000	$ 18.18
Exercisable at end of year	50,416	$ 17.48		
Vested at December 31, 2013 and expected to vest	170,000	$ 18.18		

Table of Contents

	Aggregate Intrinsic Value	Weighted Average Remaining Contractual Life (Years)
Stock Appreciation Rights outstanding at end of year	$ 720,300	8.76
Exercisable at end of year	240,097	8.75
Vested at December 31, 2013 and expected to vest	720,300	8.76

The total grant-date fair value of stock appreciation rights that vested during 2013 was approximately $0.7 million.

Employee Stock Purchase Plan (ESPP)

Under the Company s ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company s common stock on the first business day and the last business day of the relevant plan period. The initial 26-month award period will end on August 31, 2015. Each subsequent offering period will begin on March 1 or September 1.

For the year ended December 31, 2013, the fair value of stock purchase rights ranges from $16.12 to $24.65 per share on 54,995 shares estimated to be purchased during the initial award period. The fair value was estimated using the Black-Scholes-Merton option-pricing model. The Company used a weighted-average stock-price volatility ranging from 84% to 98%, expected option life assumption from 0.7 to 2.2 years and a risk-free interest rate from 0.1% to 0.4%. The Company recorded $0.5 million of stock-based compensation expense for the year ended December 31, 2013 related to the ESPP. For 2012 and 2011 there was no ESPP and as such no expense was recorded for those periods.

Stock-based Compensation Expense

Total stock-based compensation expense recognized in 2013, 2012 and 2011 was $11.1 million, $3.1 million and $3.1 million, respectively. A summary of the stock-based compensation expense recognized in the statement of operations and comprehensive loss is as follows:

	Year Ended December 31,		
	2013	2012	2011
		(in thousands)	
Research and development	$ 3,888	$ 1,173	$ 1,279
General and administrative	7,239	1,905	1,850
Total	$ 11,127	$ 3,078	$ 3,129

As of December 31, 2013, there was $47.2 million of total unrecognized compensation cost related to non-vested share-based compensation arrangements, including stock options, restricted stock, RSUs, and SARs, granted under the Plan. These costs are expected to be recognized over a weighted-average period of 3.1 years.

In 2011, the Company entered into separation agreements and releases with several of its former executives. Pursuant to these agreements, the Company immediately vested certain outstanding stock options held by these departing executives and extended the period in which the options could be exercised for a period of up to one year. As a result of these separation agreements and releases, the Company recorded a stock-based compensation expense of $0.5 million and severance and other compensation expenses of $1.3 million in 2011. There were no significant modifications to outstanding stock awards in 2013 or 2012.

4. LOSS PER SHARE

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

Table of Contents

	Year Ended December 31,		
	2013	2012	2011
	(in thousands, except per share amounts)		
Net loss	$ (111,985)	$ (121,287)	$ (2,318)
Weighted average number of shares of common stock and common stock equivalents outstanding:			
Weighted average number of common shares outstanding for computing basic earnings per share	33,850	23,602	21,599
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	33,850	23,602	21,599
Net loss per share basic and diluted	$ (3.31)	$ (5.14)	$ (0.11)

* Warrants, stock options, restricted stock units and stock appreciation rights to purchase approximately 5,158,382, 5,858,000 and 7,285,000 shares of common stock as of December 31, 2013, 2012 and 2011, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

5. EQUITY FINANCING

In April 2011, the Company sold 3.8 million shares of its common stock at $9.00 per share in an offering registered under the Securities Act. The offering generated net proceeds of $32.1 million.

In September and October 2012, the Company sold 2.0 million shares of its common stock through an At-the-Market (the 2012 ATM) offering registered under the Securities Act which generated net proceeds of $36.2 million. In December 2012, the Company sold 4.9 million shares of its common stock for $25.25 per share in an offering registered under the Securities Act. The offering generated net proceeds of $118.1 million.

In January 2013, the Company sold approximately 87,000 shares of common stock through its 2012 ATM and generated $2.1 million in net proceeds. This issuance fully exhausted the sales of common stock available under the 2012 ATM sales agreement.

On July 3, 2013, the Company entered into a second ATM offering (the 2013 ATM) allowing the Company to sell, at its option, up to an aggregate of $125 million of shares of common stock at market prices. Through December 31, 2013, the Company sold approximately 3.4 million shares under the 2013 ATM, generating $123.0 million in net proceeds and has completed the sales of common stock available under this arrangement.

6. GOVERNMENT CONTRACTS

The Company recognizes revenue from U.S. and European Union (E.U.) government research contracts during the period in which the related expenditures are incurred and presents revenue and related expenses gross in the consolidated statement of operations and comprehensive loss. In the periods presented, substantially all of the revenue generated by the Company was derived from government research contracts. In the periods presented, nearly all of the revenue the Company generated was derived from research contracts with and grants from the U.S. government. As of December 31, 2013, the Company had completed all of its contracts with the DoD except for the Marburg portion of the July 2010 and 2012 agreements for the development of therapeutics against Ebola and Marburg viruses.

Table of Contents

The following table sets forth the revenue from each of the Company s contracts with the U.S. government and other revenue for the years ended December 31, 2013, 2012 and 2011.

	Year Ended December 31,		
	2013	2012	2011
	(in thousands)		
July 2010 Agreement (*Ebola and Marburg IV*)	$ 9,064	$ 36,557	$ 42,875
June 2010 Agreement (*H1N1*)	427		3,490
May 2009 Agreement (*H1N1*)			516
August 2012 Agreement *(Intramuscular administration)*	2,791	673	
November 2012 SKIP-NMD Agreement (*DMD*)	1,263		
July 2013 Children s National Medical Center	674		
Other Agreements		99	109
Total	$ 14,219	$ 37,329	$ 46,990

The following is a description of contracts with the U.S. government:

July 2010 Contract (Ebola and Marburg Intravenous administration)

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

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

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

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

June 2010 Agreement (H1N1/Influenza)

On June 4, 2010, the Company entered into a contract with the Defense Threat Reduction Agency (DTRA) 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, or TMT, of the DoD. The period of performance for this contract ended on June 3, 2011. The Company recognized $0.4 million associated with this agreement in 2013, which was the result of an indirect rate adjustment.

May 2009 Agreement (H1N1/Influenza)

In May 2009, the Company entered into a contract with DTRA to develop swine flu drugs using its proprietary PMO and PMO*plus*® antisense chemistry. In March 2010, the contract was amended to include

Table of Contents

testing against additional influenza strains. The Company has agreed with DTRA that the key activities under this contract were completed in 2011.

August 2012 Agreement (Intramuscular administration)

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

European Union SKIP-NMD Agreement (DMD)

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

During the year ended December 31, 2013, the Company received $1.3 million in payments under the E.U. SKIP-NMD agreement, which were fully recognized as revenue during the year ended December 31, 2013 as all of the related work was performed.

July 2013 Children s National Medical Center (CNMC) Agreement

In July 2013, the Company entered into an agreement totaling $1.3 million to provide drug product to CNMC to conduct research related to the Company s DMD program. During the year ended December 31, 2013, the Company recognized $0.7 million as revenue under the agreement.

7. LONG-TERM DEBT

The Company has two loans outstanding which bear interest at 4.75%, mature in February 2027 and are collateralized by the facility the Company owns in Corvallis, Oregon. At December 31, 2013, these loans had unpaid principal balances of $1.1 million and $0.6 million, for a total indebtedness of $1.7 million. The Company incurred interest expense on these loans of $0.1 million for each the years ended December 31, 2013, 2012 and 2011.

The following table sets forth the expected future principal payments on these loans for the years shown (in thousands):

2014	$ 92
2015	98
2016	103
2017	108
2018	114
Thereafter	1,153
Total scheduled loan principal payments	$ 1,668

8. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in December 2007, January 2009 and August 2009 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.

Table of Contents

The outstanding warrants classified as liabilities are recorded at fair value on the consolidated balance sheet and are adjusted to fair value at each financial reporting period, with changes in the fair value being recorded as Gain (loss) on change in warrant valuation in the consolidated statement of operations and comprehensive loss. The fair value is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs used in the model. The following reflects the weighted-average assumptions for each of the periods indicated:

	Year Ended December 31,		
	2013	2012	2011
Risk-free interest rate	0.1%	0.2% - 0.3%	0.1% - 0.4%
Expected dividend yield	0%	0%	0%
Expected lives	0.6 - 0.7 years	1.1 - 1.6 years	1.0 - 2.7 years
Expected volatility	95.51%	139.2% - 164.1%	71.8% - 75.6%
Warrants classified as liabilities	791,508	3,127,618	4,824,827
Market value of stock at beginning of year	$ 25.80	$ 4.50	$ 12.72
Market value of stock at end of year	$ 20.37	$ 25.80	$ 4.50

The risk-free interest rate is estimated using an average of U.S. Treasury bill interest rates that correlate to the prevailing interest rates at the time of the valuation date. The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends prior to the expiration of the warrants. The expected lives are based on the remaining contractual lives of the related warrants at the valuation date. For the year ended December 31, 2013, expected volatility was estimated using a blend of calculated volatility of the Company s common stock over a historical period and implied volatility in exchange-traded options associated with the Company s common stock. Prior to January 1, 2013, expected volatility was estimated using calculated volatility of the Company s common stock over a historical period commensurate with the expected term of the option. The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these warrants by the holders.

A reconciliation of the change in value of the Company s warrants recorded as liabilities for the years ended December 31, 2013, 2012 and 2011 is as follows:

	For the Year Ended December 31,		
	2013	2012	2011
	(in thousands)		
Balance at beginning of year	$ 65,193	$ 5,446	$ 39,111
Change in value of warrants	22,027	91,938	(33,022)
Reclassification to stockholders equity upon exercise of warrants	(78,214)	(32,191)	(643)
Balance at end of year	$ 9,006	$ 65,193	$ 5,446

A summary of the Company s warrant activity with respect to 2013, 2012 and 2011 is as follows:

	For the Year Ended December 31,					
	2013		2012		2011	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Warrants outstanding at beginning of year	3,127,618	$ 8.48	4,867,477	$ 9.54	4,944,241	$ 9.48
Granted						
Exercised	(2,336,110)	7.96	(1,739,859)	11.81	(76,764)	8.34
Expired						
Warrants outstanding at end of year	791,508	$ 10.05	3,127,618	$ 8.48	4,867,477	$ 9.54

Exercisable at end of year	791,508	$ 10.05	3,127,618	$ 8.48	4,867,477	$ 9.54

Table of Contents

The following table summarizes information about warrants outstanding at December 31, 2013, all of which are exercisable.

Issue Date	Exercise Price	Outstanding Warrants at December 31, 2013	Expiration Date
1/30/2009	$ 6.96	133,637	7/30/2014
8/25/2009	$ 10.68	657,871	8/31/2014

9. SIGNIFICANT AGREEMENTS

Charley s Fund Agreement

In October 2007, Charley s Fund, Inc. (Charley s Fund), a nonprofit organization that funds drug development and discovery initiatives specific to DMD, awarded the Company a $2.45 million research grant and, in May 2009, the grant authorization was increased to a total of $5.0 million. Pursuant to the related sponsored research agreement, the grant was provided to support the development of product candidates which utilize the Company s proprietary technologies. The grant requires the Company to make mid single-digit percentage royalty payments on net sales of any such products that are successfully commercialized up to the total amount received under the grant.

As of December 31, 2013, Charley s Fund has made payments of approximately $3.4 million to the Company. Revenue associated with this research and development arrangement is recognized based on the proportional performance method, using the payment received method. To date, the Company has recognized $0.1 million as revenue, but did not recognize any revenue for the years ended December 31, 2013, 2012 and 2011. The Company does not expect to receive any incremental funding under the grant and has deferred $3.3 million of previous receipts which is anticipated to be recognized as revenue once the Company completes the remaining milestones and they are agreed to by Charley s Fund.

Isis Ercole Agreement

In May 2003, Ercole Biotechnology, Inc., or Ercole, and Isis Pharmaceuticals, Inc. or Isis, entered into a collaboration and license agreement related to RNA splicing. Research collaboration activity defined in the agreement expired in 2006. In March 2008, the Company acquired all of the stock of Ercole in exchange for 5,811,721 shares of the Company s common stock, which was valued at approximately $8.4 million, and the assumption of approximately $1.8 million in liabilities of Ercole. The Company also issued warrants to purchase its common stock (also classified as equity), which were valued at $0.4 million, in exchange for certain outstanding warrants issued by Ercole. In connection with the March 2008 acquisition, the Company assumed Ercole s obligations under the Isis agreement. This agreement contains several cross-licenses between the parties granting each party certain exclusive and nonexclusive rights under a selected set of the other parties patents and patent applications for the research, development, and commercialization of antisense therapeutics using RNA splicing with respect to certain gene targets.

Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, the Company may be obligated to make milestone payments to Isis of up to $23.4 million in the aggregate for each product developed under a licensed patent under this agreement.

As of December 31, 2013, the Company has not made, and is not under any current obligation to make, any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. The range of percentage royalty payments required to be made by the Company under the terms of this agreement is from a fraction of a percent to mid single-digit percentages. The Company believes that its DMD, Ebola, Marburg and influenza programs will not fall under the scope of this agreement and therefore will not be subject to milestone or royalty obligations under its provisions.

Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, Isis may be obligated to make milestone payments to the Company of up to $21.1 million in the aggregate for each

Table of Contents

product developed under a licensed patent under this agreement. As of December 31, 2013, Isis has not made, and is not under any current obligation to make, any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. The percentage royalty payments required to be made by Isis under the terms of this agreement is a fraction of a percent. As to any product commercialized under the agreement, the agreement will terminate on the expiration date of the last to expire licensed patent covering such product. The last to expire Sarepta owned patent covered under this agreement expires on September 9, 2014. The last Isis owned patent covered under this agreement expires on March 27, 2028. In addition, either party may terminate this agreement in the event:

- a material breach by the other party is not cured within a specified period of time; or
- the other party commences bankruptcy, reorganization, liquidation or receivership proceedings or upon the assignment of a substantial portion of the assets for the benefit of creditors by the other party with certain exceptions.

10. INCOME TAXES

As of December 31, 2013, the Company had federal and state net operating loss carryforwards of $260.9 million and $237.7 million, respectively, available to reduce future taxable income, which expire 2014 through 2033. Utilization of these net operating losses could be limited under Section 382 of the Internal Revenue Code and similar state laws based on ownership changes and the value of the Company s stock. Approximately $11.6 million of the Company s carryforwards were generated as a result of deductions related to exercises of stock options. When utilized, this portion of the Company s carryforwards, as tax affected, will be accounted for as a direct increase to contributed capital rather than as a reduction of the year s provision for income taxes. The principal differences between net operating loss carryforwards for tax purposes and the deficit accumulated during the development stage result from timing differences related to depreciation, amortization, treatment of research and development costs, limitations on the length of time that net operating losses may be carried forward, losses on changes in warrant valuation and differences in the recognition of stock-based compensation.

The Company had gross deferred tax assets of $133.6 million and $114.1 million at December 31, 2013 and 2012, respectively, primarily from U.S. federal and state net operating loss carryforwards, U.S. federal and state research and development credit carryforwards, share based compensation expense and intangibles. A valuation allowance was recorded to reduce the net deferred tax asset to zero because it is more likely than not that the deferred tax asset will not be realized.

An analysis of the deferred tax assets (liabilities) is as follows:

	December 31,	
	2013	2012
	(in thousands)	
Net operating loss carryforwards	$ 94,170	$ 85,600
Difference in depreciation and amortization	2,492	2,524
Research and development tax credits	23,599	20,012
Stock compensation	9,036	4,424
Deferred rent	2,849	265
Deferred revenue	1,219	1,133
Other	208	169
Gross deferred tax assets	133,573	114,127
Valuation allowance	(133,573)	(114,127)
Net deferred tax asset	$	$

The increase in the valuation allowance for deferred tax assets of $19.4 million for the year ended December 31, 2013, was mainly due to the increase in the net operating loss carryforwards and research and development tax credits, as well as the impact of stock option activity. The decrease in the valuation allowance for deferred tax assets of $ 2.7 million for the year ended December 31, 2012 was mainly due to the decrease

in net operating loss carryforwards.

Table of Contents

The reconciliation between the Company s effective tax rate and the income tax rate is as follows:

	Year Ended December 31,		
	2013	2012	2011
		(in thousands)	
Federal income tax rate	34.0%	34.0%	34.0%
Research and development tax credits	1.4	(0.6)	54.6
Valuation allowance	(12.4)	(7.5)	(507.4)
Permanent Differences	(8.8)	(25.9)	450.0
Other			(31.2)
Foreign rate differential	(14.2)		
Effective tax rate	%	%	%

Permanent differences affecting the Company s effective tax rate include gain (loss) on changes in warrant valuation and losses in a foreign jurisdiction. On December 31, 2012, the Company licensed certain intellectual property of Sarepta Therapeutics, Inc. to its wholly owned subsidiary, Sarepta International C.V. The parties also entered into a contract research agreement under which Sarepta Therapeutics, Inc. performs research services for Sarepta International C.V. During the year ended December 31, 2013, Sarepta International C.V. incurred $46.7 million of costs in connection with the research and development activities.

The Company s policy is to recognize interest and/or penalties related to income tax matters in income tax expense. The Company had no accrual for interest or penalties on its balance sheet at December 31, 2013 or December 31, 2012, and has not recognized interest and/or penalties in the statement of operations for 2013, 2012 or 2011. The Company has not recognized any liability for unrecognized tax benefits.

11. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement for its headquarters located in Cambridge, Massachusetts. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit to meet the requirement. The initial term of the lease agreement is for seven years with an average base rent of approximately $2.4 million per year.

In November 2013, the Company entered into an amendment of its lease agreement for its headquarters located in Cambridge. The amendment modified the original lease to add an additional 15,077 square feet to its original space, increasing its total rental space for its headquarters to 61,453 square feet. The amendment calls for additional base rate of approximately $0.5 million, subject to a 2.5% annual increase.

The Company also leases laboratory and office space in Corvallis, Oregon. Monthly base rent at the Corvallis, Oregon facility is approximately $79 thousand per month, excluding other occupancy costs, and is subject to an annual increase of 3%.

Table of Contents

Rent expense and occupancy costs under all leases totaled $3.4 million, $2.6 million and $2.5 million for 2013, 2012 and 2011, respectively. At December 31, 2013, the aggregate non-cancelable future minimum payments under leases were as follows:

	Year ending December 31, (in thousands)
2014	3,535
2015	3,807
2016	3,895
2017	3,986
2018	4,079
Thereafter	8,622
Total minimum lease payments	$ 27,924

Royalty Obligations

The Company has license agreements for which it is obligated to pay minimum royalties if the Company does not terminate the relevant agreement. The notice period to terminate these agreements is six months or less. Royalty payments under these agreements were $0.1 million for each of the years ended December 31, 2013, 2012 and 2011.

The Company is also obligated to pay royalties upon the net sales of DMD products. The royalty rates are in the low single-digit percentages for both inside and outside the United States. In addition, the Company is obligated to pay Charley s Fund a mid single-digit percentage royalty on the net sales of any product developed pursuant to the agreement with Charley s Fund up to a maximum of $3.4 million. As of December 31, 2013, the Company has not made any payments under its Isis Ercole agreement, and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. The range of percentage royalty payments required to be made by the Company under the terms of the Isis Ercole agreement, should such payments ever be made, is from a fraction of a percent to mid single-digit percentages (see Note 9 Significant Agreements).

The commercialization of other products in early stage development may require the payment of milestones or royalties upon commercialization.

Milestone Obligations

The Company has license agreements for which it is obligated to pay development milestones as a product candidate proceeds from the filing of an Investigational New Drug application through approval for commercial sale. In April 2013, the Company and the University of Western Australia (UWA) entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated. Under the terms of this agreement, UWA granted the Company an exclusive license to certain UWA intellectual property rights in exchange for up to $7.1 million in upfront and development milestone payments. In 2013, the Company recognized expense of $1.1 million relating to certain upfront payments required under the agreement within research and development in the consolidated statement of operations and comprehensive loss.

During 2012 and 2011, the Company s milestone payments were inconsequential.

Litigation

As of December 31, 2013, the Company was 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

Table of Contents

securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, in January 2014, a former consultant of the Company filed a complaint alleging breach of contract, among other claims, and seeking approximately $4 million in damages, plus certain additional fees and costs, from the Company. In addition, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 (Corban v. Sarepta et al) and January 29, 2014 (Baradanian v. Sarepta et al). The plaintiffs are alleged purchasers of Company common stock who seek to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 24, 2013 and November 12, 2013. The complaints allege that the defendants violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seek damages in an unspecified amount. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

Purchase Commitments

In the Company s continuing operations, it has entered into long-term contractual arrangements from time to time for the provision of goods and services. The following table presents noncancelable contractual obligations arising from these arrangements as of December 31, 2013:

	Year Ending December 31, (in thousands)
2014	55,641
2015	42,778
2016	42,779
2017	14,260
2018	14,260
Thereafter	
Total purchase commitments	$ 169,718

In February 2013, the Company issued two letters of credit totaling $7.3 million to a contract manufacturing vendor in connection with certain manufacturing agreements. The obligations secured by the letters of credit are fulfilled upon payment for certain minimum volume commitments that the Company expects to occur within the next twelve months. To meet the requirement of the letters of credit, the Company purchased $7.3 million in certificates of deposit with April 2014 maturity dates. If the minimum volume commitments have not occurred at that time, the letters of credit will be extended. The Company has recorded this $7.3 million as restricted investments in the consolidated balance sheet as of December 31, 2013.

12. RESTRUCTURING

In December 2011, the Company restructured its operations by reducing its workforce by 35 employees, or 28%. Restructuring charges totaling $1.1 million were recorded in 2011 and included severance and related costs. The charge included $0.5 million to research and development expense and $0.6 million to general and administrative expense.

In November 2012, the Company notified 21 Bothell, Washington based employees that they would be terminated as part of the corporate headquarters relocation to Cambridge, Massachusetts. Terminated employees were given various incentives to remain through a transition period which was completed in 2013. During 2013, the transition period was extended for a certain employee through the first quarter of 2014.

For the year ended December 31, 2012, the Company recorded a restructuring charge of $0.1 million to research and development expense and $0.1 million to general and administrative expense associated with the 2012 portion of the transition period.

For the year ended December 31, 2013, the Company recorded a restructuring charge of $0.4 million to research and development expense and $0.4 million to general and administrative expense associated with the 2013 portion of the transition period. All remaining transition costs are expected to be paid in 2014.

Table of Contents

Changes in the liability and the balance at year end related to these restructuring plans are as follows:

	Years Ending December 31,		
	2013	2012	2011
	(in thousands)		
Balance at January 1,	$ 185	$ 828	$
Restructuring charge	764	185	1,145
Payments	(905)	(828)	(317)
Balance at December 31,	$ 44	$ 185	$ 828

13. FINANCIAL INFORMATION BY QUARTER (UNAUDITED)

	2013 for Quarter Ended			
	December 31,	September 30,	June 30,	March 31,
	(in thousands)			
Revenues from license fees, grants and research contracts	$ 2,626	$ 4,168	$ 2,951	$ 4,474
Operating expenses:				
Research and development	25,076	21,087	12,984	13,762
General and administrative	10,399	8,014	7,054	6,127
Operating loss	(32,849)	(24,933)	(17,087)	(15,415)
Other income (loss):				
Interest income (expense) and other, net	45	63	(19)	237
Gain (loss) on change in warrant valuation	23,984	(17,160)	(1,945)	(26,906)
Net loss	$ (8,820)	$ (42,030)	$ (19,051)	$ (42,084)
Net loss per share basic and diluted	$ (0.23)	$ (1.24)	$ (0.60)	$ (1.32)
Shares used in per share calculations basic and diluted	37,596	33,943	31,984	31,813

	2012 for Quarter Ended			
	December 31,	September 30,	June 30,	March 31,
	(in thousands)			
Revenues from license fees, grants and research contracts	$ 7,336	$ 7,574	$ 11,207	$ 11,212
Operating expenses:				
Research and development	12,834	10,914	13,849	14,805
General and administrative	4,868	3,565	2,915	3,281
Operating loss	(10,366)	(6,905)	(5,557)	(6,874)
Other income (loss):				
Interest income and other, net	83	67	107	96
Gain (loss) on change in warrant valuation	(51,784)	(42,716)	13,488	(10,926)
Net income (loss)	$ (62,067)	$ (49,554)	$ 8,038	$ (17,704)
Net income (loss) per share basic	$ (2.36)	$ (2.17)	$ 0.36	$ (0.78)

Net income (loss) per share diluted	$ (2.36)	$ (2.17)	$ 0.35	$ (0.78)
Shares used in per share calculations basic	26,313	22,824	22,624	22,624
Shares used in per share calculations diluted	26,313	22,824	22,658	22,624

Table of Contents

EXHIBIT INDEX

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
2.1	Agreement and Plan of Merger dated June 6, 2013 between Sarepta Therapeutics, Inc., a Delaware corporation, and Sarepta Therapeutics, Inc., an Oregon corporation.	8-K12B	001-14895	2.1	6/6/13	
3.1	Amended and Restated Certificate of Incorporation.	8-K12B	001-14895	3.1	6/6/13	
3.2	Bylaws.	8-K12B	001-14895	3.2	6/6/13	
4.1	Form of Specimen Certificate for Common Stock.	10-Q	001-14895	4.1	8/8/13	
4.2	Form of Common Stock Purchase Warrant, issued on January 30, 2009.	8-K	001-14895	4.4	1/30/09	
4.3	Form of Common Stock Purchase Warrant, issued on August 25, 2009.	8-K	001-14895	4.1	8/24/09	
10.1	Employment Agreement with Patrick Iversen, Ph.D., dated July 14, 1997.	10KSB	000-22613	10.12	3/30/98	
10.2	Amendment to Employment Agreement with Patrick Iversen, Ph.D., dated December 28, 2008.	10-K	001-14895	10.5	3/15/11	
10.3	Amendment No. 2 to Employment Agreement with Patrick Iversen, Ph.D., dated January 18, 2010.	10-K	001-14895	10.6	3/15/11	
10.4	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between Sarepta Therapeutics, Inc. and Christopher Garabedian.	10-Q	001-14895	10.2	5/9/13	
10.5	Executive Employment Agreement dated January 10, 2011 by and between AVI BioPharma, Inc. and Effie Toshav.	10-Q	001-14895	10.1	5/10/11	
10.6	Executive Employment Agreement dated March 29, 2011 by and between AVI BioPharma, Inc. and Peter S. Linsley, Ph.D.	10-Q	001-14895	10.4	5/10/11	
10.7	Executive Employment Agreement dated June 13, 2011 by and between AVI BioPharma, Inc. and Edward Kaye, M.D.	10-Q	001-14895	10.4	8/8/11	
10.8	Stand Alone Stock Option Grant between AVI BioPharma, Inc. and Effie Toshav dated January 10, 2011.	10-Q	001-14895	10.2	5/10/11	
10.9	Stand Alone Stock Option Grant between the Registrant and Peter Linsley dated May 16, 2011.	S-8	333-175031	4.8	6/20/11	
10.10	Stand Alone Stock Option Grant between the Registrant and Edward Kaye dated June 20, 2011.	S-8	333-175031	4.9	6/20/11	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.11	AVI BioPharma, Inc. 2002 Equity Incentive Plan.	Schedule 14A	001-14895	Appendix A	4/11/02	
10.12	Amended and Restated Sarepta Therapeutics, Inc. 2011 Equity Incentive Plan.	8-K12B	001-14895	10.1	6/6/13	
10.13	Form of Stock Option Award Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-Q	001-14895	10.5	8/8/13	
10.14	Form of Notice of Grant of Restricted Stock under the Amended and Restated 2011 Equity Incentive Plan.	10-Q	001-14895	10.4	8/8/13	
10.15	AVI BioPharma, Inc. Non-Employee Director Compensation Policy.	8-K	001-14895	10.85	10/1/10	
10.16	Form of Indemnification Agreement.	8-K	001-14895	10.86	10/8/10	
10.17	Form of Restricted Stock Unit Award Agreement under 2011 Equity Incentive Plan.	8-K	001-14895	10.1	4/25/12	
10.18	Form of Stock Appreciate Right Award Agreement under the 2011 Equity Incentive Plan.	10-Q	001-14895	10.2	11/7/12	
10.19	Form of Senior Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.19	3/15/13	
10.20	Form of Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.20	3/15/13	
10.21	2013 Employee Stock Purchase Plan.	8-K12B	001-14895	10.2	6/6/13	
10.22	Executive Employment Agreement with Jayant Aphale, Ph.D.	10-Q	001-14895	10.1	8/8/13	
10.23	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.	10-Q	001-14895	10.3	5/9/13	
10.24	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and Sandesh Mahatme.					X
10.25	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and David Tyronne Howton.					X
10.26	Executive Inducement Stock Option Award Agreement between Arthur Krieg and Sarepta Therapeutics, Inc.					X
10.27	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan.					X

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.28	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan.					X
10.29*	Collaboration and License Agreement between Isis Pharmaceuticals and Ercole Biotech, Inc. dated May 16, 2003.	10-K	001-14895	10.78	3/16/10	
10.30*	Amended and Restated Exclusive License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc. and Sarepta International CV dated April 10, 2013.	10-Q	001-14895	10.1	5/9/13	
10.31	Agreement between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency dated May 5, 2009.	10-Q	001-14895	10.72	8/10/09	
10.32	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA1-07-C-0010), effective May 29, 2009.	10-Q	001-14895	10.74	8/10/09	
10.33	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA 1-07-C0010), effective September 30, 2009.	10-Q	001-14895	10.77	11/9/09	
10.34*	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no HDTRA 1-09-C-0046), effective March 25, 2010.	10-Q	001-14895	10.81	5/10/10	
10.35*	Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. dated June 4, 2010.	10-Q	001-14895	10.84	8/9/10	
10.36*	Modification No. PZ0001 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective March 3, 2011.	10-Q	001-14895	10.3	5/10/11	
10.37*	Modification No. P00005 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective April 13, 2011.	10-Q	001-14895	10.1	8/8/11	
10.38*	Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. dated July 14, 2010.	10-Q	001-14895	10.86	11/9/10	
10.39*	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.	10-Q	001-14895	10.1	11/7/12	
10.40*	Modification No. P00005 to Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. effective August 15, 2011.	10-Q/A	001-14895	10.3	2/15/12	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated Form	File No.	Exhibit	Filing Date	Provided Herewith
10.41*	Sponsored Research Agreement between AVI BioPharma, Inc. and Charley s Fund, Inc., effective October 12, 2007.	10-K	001-14895	10.58	3/17/08	
10.42*	First Amendment to Sponsored Research Agreement between AVI BioPharma, Inc. and Charley s Fund, Inc. dated June 2, 2009.	10-Q	001-14895	10.75	8/10/09	
10.43	Commercial Lease between Research Way Investments, Landlord, and Antivirals, Inc., Tenant, effective June 15, 1992.	SB-2	333-20513	10.9	1/28/97	
10.44	Lease Extension and Modification Agreement dated September 1, 1996, by and between Research Way Investments and Antivirals, Inc.	10-K	001-14895	10.53	3/15/11	
10.45	Second Lease Extension and Modification Agreement dated January 24, 2006 by and between Research Way Investments and AVI BioPharma, Inc.	10-Q	001-14895	10.55	8/9/06	
10.46	Real Property Purchase Agreement by and between WKL Investments Airport, LLC and AVI BioPharma, Inc., dated March 1, 2007, as amended.	10-Q	001-14895	10.61	8/9/07	
10.47	Lease Agreement between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc., dated November 23, 2011.	10-K	001-14895	10.42	3/13/12	
10.48	First Amendment to Lease Agreement dated December 22, 2011 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.43	3/13/12	
10.49	Second Amendment to Lease Agreement dated January 20, 2012 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.44	3/13/12	
10.50	Lease dated July 27, 2009 by and between BMR-3450 Monte Villa Parkway, LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.76	11/9/09	
10.51	First Amendment to Lease dated August 30, 2011 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.4	11/8/11	
10.52	Second Amendment to Lease dated January 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-K	001-14895	10.47	3/13/12	
10.53	Third Amendment to Lease dated May 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.2	8/7/12	
10.54	Lease dated October 20, 2010, by and between S/I North Creek VII LLC and AVI BioPharma, Inc.	10-K	001-14895	10.57	3/15/11	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.55	Lease Agreement dated June 25, 2013 by and between Sarepta Therapeutics, Inc. and ARE-MA Region No. 38, LLC.	8-K	001-14895	10.1	7/1/13	
21.1	Subsidiaries of the Registrant.					X
23.1	Consent of Independent Registered Public Accounting Firm.					X
24.1	Power of Attorney (contained on signature page).					X
31.1	Certification of the Company s President and Chief Executive Officer, Christopher Garabedian, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company s Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1**	Certification of the Company s President and Chief Executive Officer, Christopher Garabedian, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2**	Certification of the Company s Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

Indicates management contract or compensatory plan, contract or arrangement.

* Confidential treatment has been granted for portions of this exhibit.

** Furnished herewith.