Sarepta Therapeutics, Inc.
Form 10-K
February 26, 2015
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, 2014

Or

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

Commission file number: 001-14895

Sarepta Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

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

215 First Street

Suite 415

Cambridge, MA **02142**
(Address of principal executive offices) **(Zip Code)**
Registrant s telephone number, including area code: (617) 274-4000

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 ¨ No x

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. x

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, 2014 was approximately $1,215,422,825.

The number of outstanding shares of the registrant s common stock as of the close of business on February 23, 2015 was 41,311,512.

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 2015 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

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 often identified by words such as believe, anticipate, expect, intend, plan, will, may, estimate, could, continue, ongoing, predict, potential, likely, seek and other similar expressions, as well as variations or negatives of these words. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other forward-looking information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

our expectations regarding the timing of research, development, preclinical and clinical trial results and analyses relating to the safety profile and potential clinical benefits of our product candidates, including eteplirsen, our phosphorodiamidate morpholino oligomer (PMO) chemistries, our other PMO-based chemistries and our other RNA-targeted technology;

our expectations regarding additional data and analysis collected by us on eteplirsen, the Food and Drug Administration s (FDA) interpretation of this additional information and the impact of such data or interpretation on our new drug application (NDA) filing plans and timelines;

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

our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of our product candidates, chemistries and technologies;

the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy (DMD) and rare and infectious diseases, and their potential to treat a broad number of other human diseases;

our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;

our ability to effectively manage the clinical trial process for our product candidates on a timely basis, such as our confirmatory studies for eteplirsen;

our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance (API s) and drug product, within the time frames and quantities needed to provide our product candidates, including eteplirsen, to patients in larger scale clinical trials or in potential commercial quantities, and meet regulatory and Company quality control requirements;

the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, including with respect to the finalization of the pivotal clinical study design for eteplirsen, NDA submissions and the issuance of an Emergency Use

Authorization (EUA) for our product candidate intended to treat Marburg virus, as well as the development of our product candidates and our financial and contractual obligations;

our expectations regarding the potential markets for our product candidates;

Table of Contents

the potential acceptance of our product candidates, if introduced, in the marketplace;

the possible impact of competing products on our product candidates and our ability to compete against such products;

the impact of potential difficulties in product development, manufacturing, or the commercialization of our product candidates, including difficulties in establishing the commercial infrastructure necessary for the commercialization of eteplirsen;

our expectations regarding partnering opportunities and other strategic transactions;

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

our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;

our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization of our product candidates;

our ability to enter into contracts, including collaborations or licensing agreements, with respect to our technology and product candidates, with third parties, including government entities;

our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;

our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Office (USPTO) may take or has taken with respect to our patent claims or those of third parties, including with respect to interferences that have been declared between our patents and patent applications held by Prosensa Holding N.V. (Prosensa), which is now owned by BioMarin Pharmaceuticals, Inc., relating to eteplirsen and SRP-4053 and our expectations regarding the impact of these interferences on our business plans, including our current commercialization plans for eteplirsen and SRP-4053;

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

the impact of litigation on us, including actions brought by stockholders;

our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;

our ability to comply with applicable environmental laws and regulations;

our expectations relating to potential funding from government and other sources for the development of some of our product candidates, including those targeting Ebola and Marburg viruses;

our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements; 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 or the rules and regulations of the U.S. Securities and Exchange Commission. We caution readers not to place undue reliance on forward-looking statements. 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. Applicable risks and

Table of Contents

uncertainties include, among others, the fact that: we may be delayed or may not be able to make an NDA submission for eteplirsen or the FDA may not file the NDA or approve eteplirsen as a DMD therapeutic; we may be delayed or may not be able to comply with the FDA s requests for additional information in connection with our planned eteplirsen NDA submission; the additional information and data we collect for the eteplirsen NDA submission may not be consistent with prior data or results or may not support an eteplirsen NDA submission, filing or approval; we may be delayed in and may not be able to successfully conduct or obtain positive results in our current and planned clinical trials for eteplirsen and other product candidates in our pipeline; we may not have sufficient funds to execute on our business plans and strategy; we may not be able to obtain regulatory approvals for our product candidates in a timely manner nor achieve commercial viability; we may not be able to incorporate our PMO and other technology into therapeutic commercial products; we may not be able to successfully navigate the uncertainties related to regulatory processes; we may not be able to demonstrate acceptable levels of safety, efficacy and quality in our product candidates through our preclinical and clinical trials; compliance with environmental laws could have a negative impact on our business if we are not able to effectively manage our real estate, manufacturing and other company operations that may deal with hazardous materials; we rely on third parties to provide service, including the manufacturing of our product candidates, in connection with our preclinical and clinical development programs and commercialization plan; the pharmaceutical industry is subject to greater government scrutiny and regulation, and we may not be able to respond to changing laws and regulations affecting our industry, including any reforms to the regulatory approval process administered by the FDA or changing enforcement practices related thereto; we may not be able to obtain and maintain patent protection for our product candidates, preserve our trade secrets or prevent third parties from infringing on our proprietary rights; we may not be able to retain key personnel or attract qualified personnel; we may not be able to establish and maintain arrangements with third parties who are able to meet manufacturing needs for large-scale clinical trials or potential commercial needs within sufficient timelines or at acceptable costs; competitive products and pricing may have a negative impact on our business; there are uncertainties associated with future capital needs; we may not be able to attract sufficient capital or failure to enter into strategic relationships; the outcome of investigations and litigation and associated damages and expenses is uncertain; and those risks and uncertainties 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-targeted therapeutics for the treatment of rare, infectious and other diseases. Our highly-differentiated RNA-targeted technologies work at the most fundamental level of biology. Applying our proprietary and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying DMD drug candidates, including our lead DMD product candidate, eteplirsen, designed to skip exon 51. We are also developing therapeutics using our technology for the treatment of drug-resistant bacteria and infectious, rare and other human diseases.

Objectives and Business Strategy

We believe that our highly-differentiated and proprietary RNA-targeted 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-targeted technology platforms, organizational capabilities and resources to become a leading developer and marketer of RNA-targeted therapeutics, including for the treatment of rare, infectious and other diseases, with a diversified portfolio of product candidates. 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 potentially provide significant clinical benefits;
- further explore funding, collaboration and other opportunities to support continued development of our rare, infectious and other research and development programs; and
- leveraging RNA-targeted technology platforms and toll-like receptor (TLR) technology 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

DMD. Our lead program, with a pipeline of eight product candidates, focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD in the U.S. If we are successful in our development efforts, eteplirsen, our lead DMD product candidate, and our follow-on exon-skipping DMD candidates will address a severe and unmet medical need. We are in the process of conducting several studies with eteplirsen and our follow-on DMD candidates including:

- **Study 4658-202 (Study 202)** an ongoing U.S. open label extension study with more than 168 weeks of data collected to date (an extension of our initial Phase IIb clinical trial which was completed in 2011). After more than three years of treatment with eteplirsen, results of the 6-minute walk test (6MWT) at 168 weeks showed continued ambulation across all patients evaluable on the test, however all patients showed a decline in distance walked on this measure since the week 144 time point. In addition, a continued stability of respiratory muscle function was observed, as assessed by pulmonary function tests and measures including maximum inspiratory pressure (MIP), maximum expiratory pressure (MEP) and forced vital capacity (FVC);
- **Study 4658-301 (Study 301)** a confirmatory U.S. study, started in 2014, with a treated arm evaluating the safety and efficacy of eteplirsen in ambulatory DMD patients amenable to exon-51 skipping and an untreated concurrent control arm with patients that are

not amenable to exon-51 skipping;

Table of Contents

Study 4658-204 (Study 204) a U.S. study, started in 2014, evaluating the safety and tolerability of eteplirsen in patients with advanced stage DMD;

Study 4658-203 (Study 203) a U.S. study, expected to start in 2015, evaluating the safety and tolerability of eteplirsen in patients with early stage DMD;

SKIP-NMD Exon 53 Study a European Union (E.U.) study we are conducting in collaboration with a consortium of scientific, clinical and industrial partners in the E.U. Part I of the study, started in 2014, is a dose titration, placebo-controlled study evaluating the safety, tolerability and pharmacokinetics of SRP-4053. The focus of the planned Part II of the study will be to evaluate the safety and efficacy of SRP-4053 in patients with DMD amenable to exon 53 skipping.

In addition, we are currently working towards starting a confirmatory study in 2015 for eteplirsen, evaluating the safety and efficacy of our product candidates designed to skip exons 45 and 53. We plan to submit an NDA for eteplirsen for the treatment of DMD by mid-year 2015, subject to and pending any additional discussions with, or feedback or requests from, the FDA, our ability to satisfactorily respond to FDA requests and the favorability of additional information and data we collect to support an eteplirsen NDA submission.

Infectious Diseases. The antisense technology platform has been applied to the development of potential therapeutics for Ebola and Marburg hemorrhagic fever and pandemic H1N1 influenza viral infections. Though our original discovery and development contracts from the Department of Defense (DoD) are no longer active, we are active partners with the National Institutes of Health (NIH) National Institute of Allergy and Infectious Diseases (NIAID) for continued development of our influenza product candidate, and, if funding opportunities are secured, will continue developing the hemorrhagic fever virus countermeasures. Following encouraging preclinical results, in February 2012, we announced Phase I results for the Ebola and Marburg and influenza product candidates, which showed no clinical or toxicologic safety concerns. All three product candidates use our PMO*plus*® technology. We are currently exploring possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and influenza product candidates; however, if we do not succeed in these efforts, we will likely curtail their further development.

Discovery and Research Programs

Our discovery and research programs include collaborations with various parties and focus on developing therapeutics in rare, genetic, anti-infective, neuromuscular and central nervous system diseases. We are exploring the application of our proprietary PMO platform technology and TLR technology in various diseases including drug-resistant bacteria, DMD, Becker muscular dystrophy (Becker), progeria, adult onset pompe disease and lupus and graft-versus-host disease.

Proprietary Manufacturing Techniques

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. We have entered into certain manufacturing and supply arrangements with third parties which will in part utilize these techniques to support production of certain of our product candidates and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support a clinical or commercial supply of our product candidates.

General Corporate Information

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 (617) 274-4000. On July 12, 2012, our common stock began

Table of Contents

trading under the symbol SRPT 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 under the same symbol. 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.

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

As of December 31, 2014, we had approximately $211.1 million of cash, cash equivalents and investments, consisting of $73.6 million of cash and cash equivalents, $136.8 million of short-term investments and $0.8 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months. In addition to pursuing additional cash resources through public or private financings, we may also seek to enter into contracts, including collaborations or licensing agreements with respect to our technology, with third parties, including government entities.

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 U.S. Securities and Exchange Commission (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 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.

Lead Development Program: Pipeline of Exon-Skipping PMO Product Candidates for Duchenne Muscular Dystrophy

DMD Background

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. Females are rarely affected by the disorder. In the absence of dystrophin protein, affected individuals generally experience:

- Muscle damage characterized by inflammation, fibrosis and loss of myofibers beginning at an early age
- Muscle weakness and progressive loss of muscle function beginning in the first few years of life
- Decline of ambulation and respiratory function after the age of 7
- Total loss of ambulation in the pre-teenage or early teenage years
- Progressive loss of upper extremity function during mid to late teens
- Respiratory and/or cardiac failure in their 20s to which they typically succumb to

Table of Contents

There is currently no approved disease modifying treatment or cure for DMD in the U.S. 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.

Exon-Skipping Pipeline

Eteplirsen. Eteplirsen, our lead DMD product candidate, is an antisense PMO therapeutic in Phase III clinical development for the treatment of individuals with DMD who have an error in the gene coding for dystrophin that is amenable to skipping exon 51. Eteplirsen targets the most frequent series of mutations that cause DMD. Eteplirsen has been granted orphan drug designation in the U.S. and E.U. In 2007, the FDA granted eteplirsen fast track status and we are continuing to discuss with the FDA the possibility of expedited regulatory programs for eteplirsen. For over three years, we have been collecting data on the safety and efficacy of eteplirsen through a Phase llb open label extension study, Study 202 , which met its primary endpoint of increased novel dystrophin as assessed by the measurements taken of muscle biopsies at 48 weeks. In July 2014, we announced that at 144 weeks (i) patients evaluable on the 6MWT showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history data and (ii) a continued stabilization of respiratory muscle function was observed, as assessed by pulmonary function tests. In January 2015, we announced that at 168 weeks (i) continued ambulation across all patients evaluable on the 6MWT was observed, however, all patients showed a decline in distance walked on this measure since the week 144 time point, (ii) stability of respiratory muscle function was observed, as assessed by pulmonary function tests and (iii) good tolerability and no clinically significant treatment-related adverse events or serious adverse events reported. Please read *Management s Discussion and Analysis of Financial Condition and Results of Operations Summary and Timeline of Eteplirsen Data Disclosure* included elsewhere in this Annual Report on Form 10-K for more information. Based on FDA feedback to date, we are in the process of collecting additional information and data for eteplirsen, including conducting additional eteplirsen studies, and plan to respond to the FDA s requests in preparation for an NDA submission by mid-year 2015. We will continue to evaluate our NDA submission plans based on additional FDA discussions and as additional data become available. Please read *Overview* and *Government Regulation* for additional information.

The table below summarizes our DMD studies and the confirmatory trials we initiated in 2014 or plan to start in 2015:

Study	Duration (weeks)	US/EU	n	Status	Exon Target Treatment	DMD Population
4658-33	Single Dose	EU	7	**Completed**	Exon 51	10-17 yrs, non-amb
4658-28	12	EU	19	**Completed**	Exon 51	5-15 yrs, amb
4658-201	28	US	12	**Completed**	Exon 51	7-13 yrs, amb
4658-202	240[1]	US	12	**Data through 168 weeks**	Exon 51	7-13 yrs, amb
4658-301	48	US	160	**Dosing**	Exon 51	7-16 yrs, amb
4658-204	96	US	40	**Dosing**	Exon 51	7-21 yrs, non-amb
4658-203	96	US	40	**Dosing mid-year 2015**	Exon 51	4-6 yrs, amb
4053-101	48	EU	48	**Dosing**	Exon 53	6-15 yrs, amb
4045-804	48	EU/US	90	**TBD**	Exon 45/53	7-16 yrs, amb

Additional DMD Product Candidates. 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. Our additional seven product candidates target skipping of exons 8, 44, 45, 50, 52, 53 and 55 and are at various stages of development.

Exon 53. To support certain clinical proof of concept studies and investigational new drug (IND) -enabling activities for an exon 53-skipping therapeutic, we announced in November 2012 that we are

Table of Contents

collaborating in the SKIP-NMD Consortium 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 E.U. and the U.S. In connection with this collaboration, the consortium received an E.U. Health Innovation-1 2012 collaborative research grant (grant agreement No. 305370) 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). As of January 2015, the SKIP-NMD Consortium has advanced SRP-4053 to a Phase I study evaluating the safety, tolerability and pharmacokinetics of SRP-4053. If Part 1 is successfully completed, the study will move on to Part 2 which will study the safety and efficacy of SRP-4053. In addition to this E.U. study, we plan to further explore the safety and efficacy of SRP-4053 as part of an eteplirsen confirmatory study which we plan to start in 2015.

Exon 45. In collaboration with Children s National Medical Center (CNMC) in Washington, D.C. and the Carolinas Medical Center (CMC) in Charlotte, N.C., we have developed an exon 45-skipping product candidate. This collaboration is funded primarily through two grants, one from DoD s Congressionally Directed Medical Research Program to CNMC and the other from the National Institute of Neurological Disorders and Stroke to the CMC. This funding is intended to pursue the most promising treatments for DMD. The collaboration has supported a series of Good Laboratory Practice (GLP) toxicology studies for an exon 45-skipping drug candidate based on our PMO chemistry. SRP-4045, designed to skip exon 45, is expected to advance to the clinical stage of development in 2015 as part of the eteplirsen confirmatory trial we plan to start in 2015.

Exons 8, 44, 50, 52 and 55. Selection of lead sequences for product candidates designed to skip each of these exons are underway and we plan to continue our pre-clinical development of these product candidates in 2015. Although we were previously collaborating with the NIH for the development of an exon 50 product candidate, we mutually agreed to terminate our Collaborative Research and Development Agreement in February 2013 and we are now developing an exon 50 skipping candidate utilizing our own research and development capabilities.

Our DMD program is 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.

Development Program: Infectious Diseases

Our infectious disease therapeutic programs use our translation suppression technology and apply our proprietary PMO*plus*® chemistry platform, an advanced generation of our base PMO chemistry platform that selectively introduces positive backbone charges to improve selective interaction between the drug and its target. With the prior financial support of the U.S. government, we implemented our RNA-targeted technology in our infectious disease programs for the development of therapeutics to treat various diseases including Marburg, Ebola and influenza. Although we are no longer receiving DoD funding for our infectious disease programs, we have partnered with the NIAID, part of NIH, for clinical support in the development of our influenza therapeutic candidate. We are currently exploring possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and Influenza product candidates; however, if we do not succeed in these efforts, we will likely curtail their further development.

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, 2014, we had completed all development activities of our contracts with the U.S. government.

Table of Contents

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 Central Africa. 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. The mortality rate for Marburg virus is very high and there are currently no approved treatments beyond supportive care. The Marburg virus is classified as a Category A bioterrorism agent by the Centers for Disease Control and Prevention (CDC), and was determined to be a material threat to national security by the Secretary of Homeland Security. For Marburg virus infection, our lead product candidate is currently AVI-7288 which is designed for post-exposure prophylaxis after documented or suspected exposure to the Marburg virus. 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. In July 2012, we announced results from a non-human primate study of the efficacy of AVI-7288, which demonstrated up to 100% survival when treatment is delayed to various time points post-infection. In September 2012, we announced that the FDA 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 (JPM-MCS), in a non-human primate study, we completed an evaluation of the feasibility of an 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. In February 2014, we announced the results of the Phase I multiple ascending dose study of AVI-7288 in healthy human subjects and found no safety or toxicity concerns. In addition, in October 2014, we announced the publication of the single ascending dose safety and pharmacokinetic studies of both the Ebola and the Marburg drug candidates in the November issue of the American Society of Microbiology s journal, *Antimicrobial Agents and Chemotherapy*. The DoD contract under which AVI-7288 was being developed expired in July 2014. Please read *Management s Discussion and Analysis of Financial Condition and Results of Operations Summary and Timeline of Marburg Product Candidate Data Disclosures Government Contracts* and *Note 12, Government Contracts* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for more information.

Ebola virus. The hemorrhagic fever caused by the Ebola virus, an RNA virus similar to Marburg virus, is severe and often fatal in humans and there are currently no approved treatments for Ebola beyond supportive care. The Ebola virus is classified as a Category A bioterrorism agent by the CDC, and was determined to be a material threat to national security by the Secretary of Homeland Security. Our Ebola product candidate, AVI-7537, is a single agent designed for post-exposure prophylaxis after documented or suspected exposure to the Ebola virus. Although we believe AVI-7537 has the potential to be a therapeutic option for the Ebola virus, demonstrating survival rates between 60-80% in treated non-human primates, we suspended our development efforts with respect to our Ebola program after the DoD issued the August 2012 stop-work order and subsequently terminated the program for convenience due to the government s fiscal constraints. In October 2014, we announced the publication of the results of a single dose ascending study of our Ebola product candidate which showed no clinical or toxicologic safety concerns.

Influenza. 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 U.S. The CDC also estimated that there were up to 403,000 H1N1-related hospitalizations in the U.S. during the same time period. AVI-7100 is our lead product candidate for the treatment of influenza, previously developed under a DoD contract, and employs our PMO*plus*® technology. During our pre-clinical research, AVI-7100 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 December 2012, we entered into an agreement with NIAID to conduct a Phase I single and multiple ascending dose study with AVI-7100. The single ascending dose portion of this Phase I

Table of Contents

study, a randomized, double-blind and placebo-controlled study, was completed in the summer of 2014 and we announced results in September 2014. Results showed that AVI-7100 was well tolerated with no reported serious or clinically significant adverse events. The pharmacokinetic analysis of AVI-7100 revealed a highly similar dose-dependent profile to that of Sarepta s Ebola and Marburg PMO*plus*® drug candidates. AVI-7100 also received a favorable review from the Data and Safety Monitoring Board (DSMB), to proceed to the multiple ascending dose portion of the study. Please read *Management s Discussion and Analysis of Financial Condition and Results of Operations Government Contracts* and *Note 12, Government Contracts* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for additional information.

Discovery and Research Programs

Rare Diseases. We are currently exploring a splice-altering approach used to inhibit myostatin protein production at the messenger RNA (mRNA) level and its application possibilities in DMD and Becker using our PMO*plus*® PMO and PMO-X® technology. The results of early research appear promising with our PMOs restoring mdx mouse weight to normal mouse levels in a 10 week-old mouse and leading to increased mobility. The mdx mouse is often studied in DMD because it does not normally produce dystrophin due to a point mutation in the dystrophin gene. We are also researching possible application of our proprietary PMO technology to regulate progerin in progeria patients and alphaglucosidase in patients with adult onset pompe disease. Our early pompe research has shown our PMO technology to successfully induce exon inclusion and generate enzyme activity. In March 2014, we entered into a patent assignment agreement that secures our proprietary rights to antisense oligonucleotides (AONs) that antagonize TLR 7/8/9 and are currently furthering research and development on the application of such AONs in a variety of potential diseases including autoimmune disorders, inflammatory disorders, skin disorders, allergies and asthma. We continue to work on these discovery phase research programs and are open to collaborations with third parties.

Anti-Infectives. The rapid emergence of broad antibiotic resistance has underscored the urgent need for new paradigms in antimicrobial development. Our anti-infectives program is focused on drug-resistant bacteria identified by the CDC as urgent or serious threats to the U.S. healthcare system. Early research findings demonstrate that targeted peptide conjugated PMOs, or PPMOs, can successfully inhibit translation of essential structural genes such as acyl carrier protein (acpP), resistance proteins such as NDM-1, or those responsible for biofilm formation, which is critical for bacterial colonies to evade host immune responses or systemic antibiotics such as cepI., which is responsible for biofilm expression. Additionally, though acpP alone can be bactericidal at clinically achievable concentration, data demonstrates that co-administration of the PPMOs targeting acpP can restore antibiotic activity to clinically achievable levels in multidrug resistant Acinetobacter, E. coli, Klebsiella, and Burkholderia spp in both bench top and mouse models. Finally, we have also seen that PPMOs targeting structural genes such as acpP or cepI (responsible for biofilm expression) can penetrate and disrupt established biofilm; furthermore, the PPMOs targeting acpP can successfully kill the established bacterial colonies in Burkholderia cepacia models. We believe the results of this early research could have broad commercial applicability. We are exploring IND enabling studies now, and are open to partnership opportunities in the development of our anti-infective program.

Proprietary Platform Technology

PMO. The basis of our chemistry platform is based on PMOs. This core chemistry has been safely dosed in over 400 patients. PMOs are synthetic compounds that bind to complementary sequences of RNA by standard Watson-Crick nucleobase pairing. When targeted to mRNA, PMOs control protein translation by steric blockade. The two key structural differences between PMOs and naturally occurring RNA are that the PMO nucleobases are bound to synthetic morpholino rings (instead of ribose in RNA), and the morpholino-nucleobase subunit is linked by a phosphorodiamidate group instead of a phosphodiester group. Replacement of negatively charged phosphodiester in RNA with the uncharged phosphorodiamidate group in PMO eliminates linkage ionization at physiological pH. Because of these modifications, PMOs are resistant to degradation by plasma and intracellular enzymes. Unlike the RNA-targeted technologies of siRNAs and DNA gapmers, PMOs rely on steric blocking rather than cellular enzymatic activity to achieve their biological effects. PMOs thus operate fundamentally differently from these other well-known RNA-targeted technologies.

Table of Contents

Our PMO technologies can be used to selectively up-regulate or down-regulate the production of a target protein and correct disease-causing genetic errors by inducing targeted expression of novel proteins. Therapeutic drug candidates applying our PMO technology act upon RNA-targeted mechanisms of action through steric blockade. These mechanisms of action include, but are not limited to, pre-mRNA splice alteration and mRNA translation blockade. In effect, this enables us to design precision product candidates that intervene to create more, less, or none of certain proteins, or an analogue of the original protein that may attain greater functionality than a patient s endogenous protein.

In therapeutic applications requiring regular dosing to sustain treatment efficacy to its fullest extent, the safety of therapeutic agents is clearly of paramount concern. We believe that our PMO and PMO-based compounds significantly reduce potential for off-target effects specifically because of their demonstrated inactivity with key molecular mechanisms that are known to be toxicologically active when stimulated. Additionally, consistent with our research and development to date, we believe that PMOs do not exhibit the coagulation and immune stimulatory effects, do not stimulate TLRs or those of the RIG-I-like receptor family and do not sequester metal ions away from the catalytic centers of polymerases.

In addition to our original PMO technology, we have also developed three new PMO-based chemistry platforms. 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 PPMOs, in which cellular uptake of the PMO component, as well as its potency and specificity of tissue targeting, may be significantly enhanced.

PMOplus®. The second of these chemistries, PMO*plus*®, includes the addition of selectively introduced positive charges to PMOs. 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 such oligomers and in targeting specific cell populations.

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

We believe that our PMO-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 PMO-based technology platforms, organizational capabilities and resources to become a leading developer and marketer of PMO-based therapeutics, including for the treatment of rare and infectious diseases, with a diversified portfolio of product candidates and approved products.

Material Agreements

We believe that our RNA-targeted technology could be broadly applicable for the potential development of pharmaceutical products in many therapeutic areas. To further exploit our core technologies, we have and may continue to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities 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 Department of Health and Human Services Agreements

We previously had contracts with DoD and its agencies and the Department of Health and Human Services (DHHS) and its agencies that funded and/or supported some of our infectious diseases programs. The period of

Table of Contents

performance of our last U.S. government contract ended in July 2014. We are currently in the process of closing out our last U.S. government contract and further development of Ebola and Marburg product candidates may be limited by our ability to obtain additional funding for these programs and by the intellectual property and other rights retained by the U.S. government. For a more detailed description of our contracts with the U.S. government, please read *Management s Discussion and Analysis of Financial Condition and Results of Operations Government Contracts* and *Note 12, Government Contracts* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K. For a description of the risks we face relating to such rights of the government, please read *Risk Factors Risks Relating to Our Business.* Any contracts that we may enter into with the government may be subject to renegotiation or termination at the election of the government.

University of Western Australia

In November 2008, we entered into an exclusive license agreement with the University of Western Australia (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 (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.

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 aggregate based on successful achievement of certain regulatory and commercial milestones relating to eteplirsen and up to five additional product candidates and may also be required to pay royalties of 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. As of December 31, 2014, we are not under any current obligation to make royalty payments to UWA until achievement of the first 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 November 2030, however, pending patents could result in a later expiration date.

Strategic Alliances

Isis Ercole Agreement

In May 2003, Ercole Biotechnology, Inc. (Ercole) and Isis Pharmaceuticals, Inc. (Isis), entered into a collaboration and license agreement related to RNA splicing. Research collaboration activity defined in the

Table of Contents

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 (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, 2014, 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.

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, 2014, Isis has not made and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. 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 expired 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. (Charley s Fund), a nonprofit organization that funds drug development and discovery initiatives specific to DMD, awarded us a research grant of approximately $2.5 million 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, 2014, 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. We have deferred $3.3 million of previous receipts which are anticipated to be recognized as revenue upon resolution of outstanding performance obligations.

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

Table of Contents

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, 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-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 be terminated by its own terms at the completion of the research being sponsored by Charley s Fund. Our 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 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 state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. 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 currently do not have 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 APIs, for our product candidates.

For our DMD program, we are working with our existing manufacturers to increase our API production capacity from mid-scale to large-scale. During 2015, 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 2015 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.

Table of Contents

Manufacturers and suppliers of product candidates are subject to the FDA s current Good Manufacturing Practices (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 U.S. 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 U.S. ourselves or enter into arrangements with other pharmaceutical or biotechnology companies for the marketing and sale of our products outside the U.S. either globally or on a country-by-country basis.

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.

Our patents and patent applications are directed to our product candidates as well as to our PMO-based technology platforms. We seek patent protection for certain of our product candidates and proprietary technologies by filing patent applications in the U.S. and other countries. As of February 4, 2015, we owned or controlled approximately 355 U.S. and corresponding foreign patents and 235 U.S. and corresponding foreign patent applications. We intend to protect our proprietary technology with additional filings as appropriate.

Our product candidates and our technology are primarily protected by composition of matter and use patents and patent applications. Currently, our clinical product candidates include AVI-7288 (Marburg), AVI-7100 (influenza) and eteplirsen (DMD). We own issued patents covering composition and methods of use for AVI-7288 in the U.S. We have exclusively licensed patents covering composition of matter and methods of use for eteplirsen in the U.S. and Europe. Additionally, we have pending patent applications 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 U.S., 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)
Antibacterial	2018 (patents) 2035 (patent applications)
Other rare diseases	2025 (patent applications) 2034 (patent applications)
Other targets and programs	2019 (patents) 2032 (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.

Table of Contents

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 multiple federal trademark registrations in the United States including Sarepta®, Sarepta Therapeutics®, PMO*plus*®, PMO-X® , Let s Skip Ahead® and the Sarepta Therapeutics logo. We have multiple pending trademark applications in the United States including: The Promise of Science, Realized ; Transformation, Within Reach ; and Turning Discovery Into Recovery . In the E.U., we have trademark registrations for Sarepta®, the Sarepta Therapeutics logo, Transformation, Within Reach® and Turning Discovery Into Recovery®.

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 U.S. 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;
- 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 U.S. 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. 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. For example, we are aware of certain claims that our competitor Prosensa has rights to in the United States that, if granted, may provide the basis for Prosensa or other parties that have rights to these claims to assert that our drug candidates, eteplirsen and/or SRP-4053, infringe on such claims. In 2014, the Patent Trial and Appeal Board (PTAB) of the USPTO declared various patent interferences between certain patents held by Sarepta under a license from the UWA and patent applications held by Prosensa under license from Academisch

Table of Contents

Ziekenhuis Leiden (AZL) related to exon 51 and exon 53 skipping therapies designed to treat DMD. Patents held or licensed to Sarepta and included in these interference proceedings are presumed valid by statute for the duration of these proceedings and any appeals. These interferences do not currently change our plans to submit an NDA for eteplirsen, continue with our clinical development plans for eteplirsen and SRP-4053 or our ability to launch eteplirsen commercially if it is approved by the FDA under an accelerated approval pathway, however, if final resolution of these interferences and related appeals, if any, are not in our favor, our current business, development and commercialization plans for eteplirsen and SRP-4053 may be negatively impacted. For details on and risks related to the interferences that PTAB has declared involving our patents, please read *Risk Factors Risks Relating to Our Business Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our product candidates, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties.*

The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain. For example, Prosensa has rights to European Patent No. EP 1619249. We opposed this patent in the Opposition Division of the European Patent Office (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 candidate, 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.

In addition to government, court and regulatory patent decisions, changes in either the patent laws or in interpretations of patent laws in the U.S. 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, exportation and marketing of our products are subject to extensive regulation by governmental authorities in the U.S. and in other countries. In the U.S., 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, first and foremost, submit clinical data providing substantial evidence of safety and efficacy of the product for its intended use, as well as detailed information on product composition, its manufacture and controls 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.

Table of Contents

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

- preclinical laboratory tests and animal toxicity testing;
- submission of an IND application for conducting human clinical testing to the FDA, 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;
- the submission to the FDA of an NDA;
- satisfactory completion of an FDA inspection of the commercial manufacturing facilities at which the drug substance and drug product are made to assess compliance with cGMP;
- satisfactory FDA audit of the clinical trial site(s) that generated the pivotal safety and efficacy data included in the NDA and also potentially the nonclinical manufacturing site(s) in the form of a pre-approval inspections; and
- FDA review and approval of the NDA.

Preclinical studies may include laboratory evaluations of the product chemistry, pharmacology, toxicity and formulation, as well as animal studies to assess the pharmacokinetics, metabolism, biodistribution and toxicity 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, manufacturing information, analytical data and a proposed first in human clinical trial protocol are submitted to the FDA as part of the IND, which must become effective before clinical trials may be initiated. The IND will become effective approximately 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the supportive data, the design, particularly regarding potential safety issues of conducting the clinical trial as described in the protocol. In this situation, the trials are placed on clinical hold and the IND sponsor must resolve any outstanding FDA concerns before clinical trials can proceed.

Clinical trials involve the administration of the product candidate to healthy volunteers or patient participants under the supervision of a qualified principal investigator. Clinical trials are conducted under protocols detailing the objectives of the study, the administration of the investigational product, study procedures, parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as a submission to the IND. Clinical trials must be conducted in accordance with the FDA s Good Clinical Practice (GCP) requirements and federal and state laws and regulations protecting study subjects. Further, each clinical trial must be reviewed and approved by the Institutional Review Board (IRB) at or servicing each institution in which the clinical trial will be conducted. The IRB will consider, among other things, rationale for conducting the trial, clinical trial design, participant informed consent, ethical factors, the safety and rights of human subjects and the possible liability of the institution. The FDA can temporarily or permanently halt 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 a particular site be halted, either temporarily or permanently, for failure to comply with the GCP or IRB s requirements, or may impose other conditions.

Clinical trials typically are conducted in three sequential drug development phases (Phases I, II and III) prior to approval, a portion of these phases may overlap. A fourth post-approval phase (Phase IV) may include additional clinical studies. A general description of clinical trials conducted in each phase of development is provided below. However, the number of study subjects involved in each phase of drug development for rare diseases can be significantly less than typically expected for more common diseases with larger patient populations:

Phase I. Phase I clinical trials involve the initial introduction of the drug into human subjects. These studies are usually designed to determine the safety of single and multiple doses of the compound and

Table of Contents

determine any dose limiting toxicities or intolerance, as well as the metabolism and pharmacokinetics of the drug in humans. Phase I studies usually involve less than 100 subjects and are conducted in healthy adult volunteers unless the drug is toxic (*e.g.,* cytotoxics) in which case they are tested in patients.

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

Phase III. If an investigational drug is found to be potentially effective and to have an acceptable safety profile in early phase studies, larger Phase III clinical trials are conducted to confirm clinical efficacy, dosage and safety in the intended patient population, which may involve 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 which establish the safety and efficacy of the drug for a specific indication are required for approval of an NDA.

Phase IV. Phase IV trials are clinical studies conducted after the FDA has approved a product for marketing. Typically there are two forms of Phase IV trials: those that are conducted to fulfill mandatory conditions of product approval and those that are voluntarily conducted to gain additional experience from the treatment of patients in the intended therapeutic indication. The mandatory studies are used to confirm clinical benefit in the case of drugs approved under the accelerated approval regulations or to provide additional clinical safety or efficacy data for full approvals. Failure to promptly conduct and complete mandatory 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 U.S. 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 unless the submission is for an Orphan Indication. The FDA assesses all NDAs submitted for completeness before it accepts them for filing and review. FDA may request additional information before accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the current NDA review goals mandated under the Prescription Drug User Fee Act (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 or 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 defines 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. 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. If the FDA s evaluation of the NDA and the commercial manufacturing procedures and facilities is not favorable, the FDA may refuse to approve the NDA. The FDA may also refer an application to an advisory committee, typically comprised of a panel of expert clinicians and researchers, for review, evaluation and a recommendation as to whether the application should be approved for the proposed indication. The FDA is not bound by, but typically follows, the recommendations of the advisory committee.

A sponsor may also seek designation of its drug candidates under programs designed to accelerate the FDA s review and potential approval of NDAs. For instance, a sponsor may seek FDA designation of a drug

Table of Contents

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 and 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. Eteplirsen was granted fast track status in 2007 and both AVI-7288 and AVI-7537 were granted fast track status in September 2012.

The Food and Drug Administration Safety and Innovation Act (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 the sponsor companies and the FDA with greater flexibility and 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 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 of a drug that treats a serious condition and generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality (IMM) that is reasonably likely to predict an effect on IMM or other clinical benefit (i.e., an intermediate clinical endpoint). Approvals of this kind typically include requirements for appropriate post-approval Phase IV clinical trials to confirm clinical benefit. 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 multiple meetings with the FDA during 2013 and 2014 to discuss the most appropriate regulatory pathway for early registration/approval of eteplirsen based on the Phase IIb data. In addition, we also had discussions with the FDA to finalize the confirmatory study designs for a potential accelerated approval for eteplirsen. Based on feedback from these meetings, we will continue to pursue the most appropriate regulatory pathway 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 guidance entitled Expedited Programs for Serious Conditions Drugs and Biologics in May 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, if a drug candidate demonstrates a significant benefit over existing therapy, it may be eligible for priority review, which means it will be reviewed within a six-month timeframe from the date a complete NDA is accepted for filing.

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.

Table of Contents

Holders of an approved NDA are required to:

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

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 (U.K.) and intend to submit for marketing approval in countries other than the U.S. 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 where conducting controlled clinical trials to determine efficacy may be unethical or unfeasible, such as infection caused by exposure to various hemorrhagic fever viruses, alternative methods for demonstrating the safety and efficacy of product candidates may be applied. Under regulations issued by the FDA in 2002 (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 filo virus 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 was provided by the FDA Draft Guidance For Industry Product Development Under the Animal Rule released in May 2014.

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 (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

Table of Contents

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.

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

clarifying the FDA s authority 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 DHHS 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; and

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.

Orphan Drug Designation and Exclusivity

In the U.S., 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 U.S., or more than 200,000 individuals in the U.S. for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that drug. In the U.S., 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 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 generally entitled to an orphan drug exclusivity period, which means the FDA may not grant approval to any other application to market the same chemical entity 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 orphan 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 applied to products developed for adult use and 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 be added to previously granted exclusivity, such as orphan drug exclusivity and new chemical entity exclusivity. 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 U.S.

Table of Contents

In Europe, Orphan Medicinal Product designation is considered by the European Medicines Agency (EMA) 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 E.U., including compounds for serious and chronic conditions that 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 designation are significant, including up to 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 extension 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 E.U.

Ex-U.S. 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 medicinal products. Outside of the U.S., 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, applications for 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.

Other Regulatory Requirements

In addition to regulations enforced by the FDA and foreign authorities relating to the clinical development and marketing of products, we are or may become subject to regulation under the Occupational Safety and Health Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and other present and potential future foreign, federal, state and local laws and regulations. Although we believe that we are in material compliance with applicable environmental laws that apply to us, we cannot predict whether new regulatory restrictions will be imposed by state or federal regulators and agencies or whether existing laws and regulations will adversely affect us in the future.

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 U.S., 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 U.S. to fundamental changes. There have been, and we expect there will continue to be, legislative and regulatory

Table of Contents

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;
- 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.

Table of Contents

DMD Program Competition. Currently, no disease-modifying product has been granted full approval for the treatment of DMD and no product is commercially available outside the European Economic Area (EEA). 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, (GSK), in January 2014, and PTC Therapeutics, Inc., (PTC), have product candidates in development for the treatment of DMD. Nippon Shinyaku also has product candidates in early clinical development for exon skipping for multiple exons but we are not aware of any public release of clinical data to date.

PTC has a small molecule candidate, ataluren, which targets nonsense mutations in development. The European Commission granted conditional marketing authorization for ataluren for the treatment of a subset of DMD patients in August 2014. Ataluren uses a distinct scientific approach that addresses a different genotype of DMD patients compared to eteplirsen. Therefore, we do not believe ataluren is appropriate for the treatment of DMD patients that are amenable to exon-skipping therapy. Additionally companies such as Santhera, Summit, Pfizer and Tivorsan have unique product candidates in different stages of development or approval in DMD which we believe could be seen as complementary to exon skipping and not a direct replacement of our clinical candidates at this time.

Prosensa submitted the first module for an NDA regulatory filing for its exon 51 skipping product candidate, drisapersen, and has announced its plans to complete the submission by end of first quarter of 2015. In November 2014, BioMarin Pharmaceuticals, Inc. announced its intention to acquire Prosensa and in January 2015, announced the completion of a tender offer for Prosensa s shares. The Prosensa 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 exon 51. 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 6MWT. In September 2013, GSK and Prosensa announced that the Phase III clinical study of drisaparsen did not meet the primary endpoint of a statistical significant improvement in the 6MWT 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.

Additionally, several companies have recently entered into collaborations or other agreements for the development of product candidates, including mRNA, 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, Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam Pharmaceuticals, Inc., (Alnylam), Moderna Therapeutics, Inc., Summit plc and Oxford University.

Hemorrhagic Fever Virus Program Competition. No specific treatment has been proven effective, and no approved vaccine currently exists for treatment or prophylaxis of either Ebola virus or Marburg virus. These agents must be tested extensively in animals and meet strict government regulations. Investigational compounds can only be tested for efficacy in humans during outbreak situations such as the current Ebola outbreak in West Africa that began in early 2014. The exigency and scale of the 2014 Ebola virus outbreak in West Africa has accelerated the development of both treatments and vaccines for Ebola. Several vaccine candidates have reached the clinical development stage and are actively being tested for population safety and potentially efficacious immunoprotective effect as a prophylactic agent. These include vaccine candidates sponsored by the biotechnology industry and also candidates in development by U.S. government agencies (*e.g.*, the National Institute of Allergy and Infectious Diseases and the DoD). The government is also supporting early

Table of Contents

stage research on therapeutics against hemorrhagic fever viruses, including broad-spectrum therapeutics. Among the most advanced therapeutic candidates that might have utility in combating Ebola virus, are candidates being developed by the Tekmira Pharmaceutical Corp., Toyama Chemical Co. LTD, BioCryst Pharmaceuticals Inc., and Mapp Bioharmaceutical Inc. with the support of the US government. Additionally, investigation of the use of convalescent plasma containing Ebola virus antibodies as a treatment modality, as well as for the potentially efficacious repurposing of drugs not intended to treat Ebola virus, remain an ongoing pursuit by the biopharmaceutical industry and several national government agencies.

Influenza Program Competition. Currently, there are three therapeutic products for influenza that have received market approval from the FDA and are recommended for use in the U.S. These are: (1) oseltamivir (Tamiflu), a Roche Holding and Gilead product; (2) zanamivir (Relenza), a GSK product; and (3) peramivir (Rapivab), a BioCryst Pharmaceuticals Inc. product. In addition to these products, Biota Pharmaceuticals and Daiichi Sankyo s laninamivir was launched in 2010 in Japan. Currently, funding from the DHHS Biomedical Advanced Research and Development Authority is helping support clinical trials of, Romark Laboratories nitazoxanide. In addition, other companies have influenza therapeutic compounds against viral and host targets in various stages of development, including Vertex Pharmaceutical and Janssen Pharmaceutical s VX-787, Biota Pharmaceutical s laninamivir, Autoimmune Technologies flufirvitide-3, Ansun BioPharma s fludase, and 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. Several additional companies, including Crucell Inc., Celltrion Inc., Visterra Inc. and Genentech Inc. are also currently developing monoclonal antibodies for use against various influenza strains to confer passive or active immunotherapeutic response. 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 clinical trials. 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 Competition. We believe that other biotechnology and pharmaceutical companies share a focus on RNA-targeted drug discovery and development. Competitors with respect to our RNA-targeted technologies include, but are not limited to, Alnylam, Tekmira Pharmaceuticals Corp., Isis, Prosensa, Sanofi, Synthena AG and Santaris Pharma A/S.

Research and Development

Our discovery, research and development programs span various disease targets. 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 trials and manufacturing activities. We do not maintain or evaluate, and therefore do not allocate, internal research and development costs on a project-by-project basis. As a result, a significant portion of our research and development expenses are not tracked on a project-by-project basis, as the costs may benefit multiple projects.

Table of Contents

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 periods indicated:

	For the Year Ended December 31,		
	2014	2013	2012
	(in thousands)		
Development programs			
DMD	$ 43,710	$ 43,511	$ 12,181
Infectious diseases	3,011	5,701	22,956
Internal research and development expenses	47,510	23,697	17,265
Total research and development expenses	$ 94,231	$ 72,909	$ 52,402

Employees

As of December 31, 2014, we had 204 employees, 84 of whom hold advanced degrees. Of these employees, 133 are engaged directly in research and development activities and 71 are in general and administration. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good.

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

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

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

Table of Contents

restricted, or unable to further develop our active and other product candidates and successfully obtain approvals needed to market them.

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

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

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

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

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

We are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the United States or the European Union, our business and results of operations could be materially adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the United States and the European Union for the time periods specified

Table of Contents

above, we would not be able to exclude other companies from manufacturing and/or selling products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot guarantee that another company will not receive approval before we do of an orphan drug application in the United States or the European Union for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our drug candidates for which we plan to file for orphan designation and status. If that were to happen, our orphan drug applications for our product candidate for that indication may not be approved until the competing company s period of exclusivity has expired in the United States or the European Union, as applicable. Moreover, we cannot guarantee that another company will not receive approval before we do to market a product candidate that is granted orphan drug status in the United States or the European Union for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our drug candidates for which we plan to file a new drug application or marketing authorization application. If that were to happen, any pending new drug application or marketing authorization application for our product candidate for that indication may not be approved until the competing company s period of exclusivity has expired in the United States or the European Union, as applicable. Further, application of the orphan drug regulations in the United States and Europe is uncertain, and we cannot predict how the respective regulatory bodies will interpret and apply the regulations to our or our competitors product candidates.

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

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

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

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

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the United States, approvals and oversight from federal (e.g. FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the United States or other countries is not permitted until we obtain

Table of Contents

the required approvals from the applicable regulatory authorities. Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates, including eteplirsen, on an accelerated approval (e.g. under FDASIA) or any other basis, in any jurisdiction, including in the United States, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

Our preclinical, clinical, Chemistry, Manufacturing and Controls (CMC) and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for submissions, filings or approvals. The FDA could disagree with our beliefs, interpretations and conclusions regarding data prior to or as part of an NDA submission, including any of the additional information and data we are currently collecting for eteplirsen, or other product candidates, and may delay, reject or refuse to file our planned NDA submission until we meet their additional requirements, if ever. Even if we meet such requirements and our NDA is accepted for review or filed, the FDA could still deny approval of eteplirsen, or other product candidates, based on their review of the data or other factors.

The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and novel surrogate endpoints, such as dystrophin measures, is uncertain due to the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. For example, 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. As a result of uncertainty in the approval process, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned INDs and NDAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (*e.g.*, additional patient muscle biopsies and dystrophin analysis), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, even if initially accepted, regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process. Furthermore, we are currently in the process of collecting additional eteplirsen data and analysis, some in direct response to FDA requests. For example, the FDA has expressed concerns with dystrophin as a surrogate endpoint and has requested an independent assessment of dystrophin positive fibers measured in our eteplirsen Phase IIb study. The FDA has also requested matched natural history to better evaluate the ongoing clinical results of our eteplirsen 201/202 study. We plan to discuss these additional data with the FDA once collected and include them in our planned NDA submission. Material inconsistencies between our existing data and analysis and the new and additional data we are collecting, including the independent assessment of dystrophin positive fibers, safety data, matched natural history and data from a fourth biopsy, could delay or otherwise negatively impact our planned eteplirsen NDA submission. Additionally, the FDA may determine, after evaluating the totality of our existing data and analysis, the additional data and analysis we are generating and any material inconsistencies between them that such data and analysis do not support an NDA submission, filing or approval.

We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Responding to requests from regulators and meeting requirements for submissions, filings and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third-party vendors and associates may be complicated by our own limitations and those of the parties we

Table of Contents

work with. For example, changes to CMC processes for the production of eteplirsen may require coordination with our third-party manufacturers, which may or may not be limited in their abilities to execute such regulatory requests. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including related to clinical trial design and the timing of NDA filings.

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

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

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive preclinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future preclinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in preclinical and early clinical trials does not ensure that the subsequent trials we plan to conduct will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld. For example, in 2012, we completed Study 201, a U.S.-based Phase IIb 12-person clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. Following completion of this study, we initiated Study 202, an ongoing open label extension study with the same participants from Study 201. These trials were initiated, in part, to further demonstrate efficacy and safety, including the production of dystrophin, and explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. While Studies 201 and 202 met their primary endpoints of dystrophin production based on the measurements taken at weeks 24 and 48, respectively, and six-minute walk test results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot provide assurances that data from the ongoing open label extension study will continue to be positive or consistent through the study periods. For example, on July 10, 2014, we announced the results for week 144 in Study 202, which showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history; however, the decline on the six-minute walk test from baseline, although in prior study results was below 5%, was measured at approximately 8.5%. Additionally, on January 12, 2015, we announced results for week 168 in Study 202, which showed continued ambulation across all patients evaluable on the test, however all patients showed a decline in distance walked on this measure since the week 144 time point. If the data from the confirmatory studies for eteplirsen do not produce the safety and efficacy data required by regulatory authorities for an NDA submission, filing or approval, we may need to continue working with the FDA on the design and subsequent execution of any further studies or analysis we plan to conduct or that may be required for the approval of eteplirsen or our other DMD product candidates. For example, in October 2014, we received meeting minutes from a Type B Pre-NDA meeting that took place in September 2014 in which the FDA provided updated guidance regarding the information to be provided as part of, or at the time of, our planned NDA submission for eteplirsen. The guidance stated that the FDA was requiring additional data as part of the

Table of Contents

NDA submission, including the results from an independent assessment of dystrophin images and the 168 week clinical data from Study 202. Additionally, the guidance requested more specific data, such as a minimum duration of safety in new patients exposed to eteplirsen, patient-level natural history data to be obtained by us from independent academic institutions and MRI data from a recent study conducted by an independent group. The FDA also indicated that further discussion would be needed to determine what would constitute a complete NDA submission. The results of the additional data we are collecting in response to the FDA s request may not be consistent with prior results or may not support our planned NDA submission.

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

We do not currently have the internal ability to undertake the manufacturing process for our product candidates in the quantities needed to conduct our research and development programs, supply clinical trials or meet commercial demand. We therefore rely on, and expect for the foreseeable future to continue relying on, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), drug substance (API) and drug product, as well as to perform additional steps in the manufacturing process, such as the filling and labeling of vials and storage of our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of our product candidates which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available product candidates or materials.

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

We have not engaged or contracted with all the third parties needed for the production of materials and APIs for any of our product candidates, including eteplirsen, in quantities sufficient for their potential commercial demand or for multiple large-scale clinical trials. In light of the limited number of third parties with the expertise to produce our product candidates, and the underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our product candidates. Further, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the regulatory approval process and potential commercialization. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties which could have a material adverse effect on our business prior to and after commercialization of any of our product candidates.

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

Our contract manufacturers are required to produce our materials, APIs and drug products under current cGMP standards. We and our contract manufacturers are subject to periodic unannounced inspections by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations and corresponding foreign standards. We do not have control over a third-party

Table of Contents

manufacturer s compliance with these regulations and standards. In addition, changes in cGMP standards could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our product candidates in a compliant manner on the schedule we require for clinical trials or for potential commercial use. The failure to achieve and maintain high quality standards, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in patient injury or death or product recalls. Any difficulties or delays in our contractors manufacturing and supply of product candidates, or any failure of our contractors to maintain compliance with the applicable regulations and standards could increase our costs, make us postpone or cancel clinical trials, prevent or delay regulatory approvals by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, result in the termination of the development of a product candidate, or have our product candidates recalled or withdrawn from use.

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

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for larger and later stage clinical trials for our product candidates, including eteplirsen, and potential commercialization, we are working to increase the manufacturing capacity and scale up production of some of the components of our drug products. During 2015, we will continue to increase material and API production capacity to provide the drug product needed for additional eteplirsen trials and studies for our other product candidates (including a placebo-controlled study planned for one or more of our follow-on exon product candidates) and any planned subsequent commercialization, on an accelerated or other pathway. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third-party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory standards or is cost-effective, or in a time frame required to meet our timelines for clinical trials, potential commercialization and other business plans, or at all. cGMP and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of a product candidate itself or the product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release product and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our analytical methods or demonstrate adequate purity, stability or comparability of the product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our analytical methods or to demonstrate adequate purity, stability or comparability, the development of our product candidates and regulatory approval or commercial launch for any resulting drug products may be delayed, which could significantly harm our business.

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

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

We have historically relied on U.S. government contracts and awards to fund and support certain development programs, including our Ebola and Marburg programs. The July 2010 DoD contract providing

Table of Contents

funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits and other government requirements successfully, the government may withhold some or all of the currently outstanding amounts owed to us.

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

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

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

recruit and retain employees, consultants or contractors with the required level of expertise;

recruit and retain sufficient patients needed to conduct a clinical trial:

Participant enrollment and retention is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;

timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations (CROs) involved in the clinical trial:

Negotiating contracts and other related documents with clinical trial parties and iIRBs, such as informed consents, CRO agreements and site agreements, can be subject to extensive negotiations that could cause significant delays in the clinical trial process. In addition, terms may vary significantly among different trial sites and CROs and may subject the Company to various risks;

ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;

manage or resolve unforeseen adverse side effects during a clinical trial;

conduct the clinical trials in a cost effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and

execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

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

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

We incurred an operating loss of $133.8 million for twelve months ended December 31, 2014. Our accumulated deficit was $679.0 million as of December 31, 2014. Substantially all of our revenue to date has

Table of Contents

been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any material revenue from product sales and have generally 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. We anticipate that our expenses will increase substantially if and as we:

- continue our research, preclinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- acquire or in-license other product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- 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 with third parties for one or more of our programs, complete development of our product candidates, obtain regulatory approvals and market our approved products, if any. It is uncertain when, if ever, we will become profitable and if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the Company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

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

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

distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

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

Table of Contents

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. For example, on April 29, 2014, we sold 2,650,000 shares of our common stock in an underwritten public offering at a price to the public of $38.00 per share. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than preclinical collaborations with academic or 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. If we were to have such a strategic relationship, such third party may require us to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or to grant licenses on terms that may not be favorable to us.

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

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

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

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

Table of Contents

such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

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

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

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

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

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

If we are unable to effectively manage our growth, execute our business strategy and implement compliance controls and systems, the trading price of our common stock could decline. Any failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

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

For example, although there was no material error in our consolidated 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. We designed and implemented controls to address the material weakness that was identified. However, we cannot provide assurances that material weaknesses in our internal control over financial reporting will not be identified in the

Table of Contents

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 consolidated financial statements and cause us to fail to meet our reporting obligations or prevent fraud, which could cause the trading price of our common stock to decline.

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

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

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

Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies and 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. Even when our 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.

We may not be able to obtain and maintain patent protection for our product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise might not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the PTAB) of the USPTO declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, UWA) and patent applications held by Prosensa (under license from Academisch Ziekenhuis Leiden, AZL) related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta s/UWA s U.S. Patent Nos. 7,807,816 and 7,960,541, both covering eteplirsen, as interfering with Prosensa s/AZL s U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta s/UWA s U.S. Patent No. 8,455,636, covering SRP-4053, as interfering with Prosensa s/AZL s U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the exon 51 skipping therapies that are

Table of Contents

the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta s/UWA s U.S. Patent No. 8,486,907, which covers certain methods of using eteplirsen, as interfering with Prosensa s/AZL s U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB, which we filed in November 2014, requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta s/UWA s U.S. Patent No. 8,455,636 and Prosensa s/AZL s U.S. Application No. 14/248,279. If final resolution of the interferences and related appeals, if any, are not in our favor, then the Sarepta/UWA patents and any other Sarepta patents or applications also found to be interfering may be invalidated, and as a result, we may not have any patent-based exclusivity available for our product candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interferences and related appeals, if any, are not in our favor, then the USPTO may issue the Prosensa/AZL patent applications resulting in the grant of one or more patents that may provide a basis for Prosensa to allege that our drug candidates, eteplirsen and/or SRP-4053, infringe such patents. These interferences may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, Prosensa may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the United States and in other jurisdictions. We are also aware of certain pending and granted claims that have been issued to Prosensa in Japan and certain other countries outside of Europe and the United States that may provide the basis for Prosensa or other parties to assert that eteplirsen infringes on such claims. 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.

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. Additionally, jurisdictions other than the United States might have less restrictive patent laws than the United States, giving foreign competitors the ability to exploit these laws to create, develop and market competing products. 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.

The full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc*., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics, Inc*., the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the

Table of Contents

decision on patents for other isolated natural products is uncertain and, as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

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

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

Any of these events could substantially harm our potential earnings, financial condition and operations. Prosensa, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. Prosensa has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office (EPO), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of eteplirsen in Europe would infringe on such patent. Both we and Prosensa have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other s briefs. Prosensa recently filed arguments with the EPO in response to Sarepta s previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial effect on our business and leaves open the possibility that Prosensa or other parties that have rights to such patent could assert that our drug candidate, eteplirsen, infringes on such patent. The timing and outcome of 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 existing patent claims Prosensa is pursuing in the United States, including those involved in the interferences declared by the USPTO in July 2014 and September 2014 and discussed in these risk factors, and others that it has or is pursuing in other countries, that where granted may provide the basis for Prosensa or other parties to assert that commercialization of eteplirsen and certain other of our product candidates would infringe on such claims.

The DMD patent landscape is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our

Table of Contents

current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

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

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

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

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

develop safer or more effective products;

implement more effective approaches to sales and marketing;

develop less costly products;

obtain regulatory approval more quickly;

have access to more manufacturing capacity;

develop products that are more convenient and easier to administer;

form more advantageous strategic alliances; or

establish superior intellectual property positions.

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

We currently have no products that have been approved for commercial sale; however, the current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of any products in the future, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies,

Table of Contents

our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for any of our product candidates. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

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

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

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

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

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

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

Table of Contents

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

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

Broad market and industry factors may seriously affect the market price of a company s 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;

Table of Contents

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

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

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

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

prohibition on stockholder action by written consent;

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

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

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

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

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

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

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and 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, 2014, there were 41.3 million shares of common stock outstanding and outstanding awards to purchase 5.3 million shares of common stock under various incentive stock plans. Additionally, as of December 31, 2014, there were 1.8 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and 0.6 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to

purchase common stock, perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Table of Contents

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 Cambridge, Massachusetts, Andover, Massachusetts and Corvallis, Oregon are 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, Cambridge, MA 02142	88,329	January February 2021	Laboratory and office space	Corporate headquarters
100 Federal Street, Andover, MA	60,000	N/A facility is owned	Manufacturing and office space	Primarily manufacturing space**
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. (Perpetua), agreed to lease approximately 25,000 square feet of the building until March 2017. 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.

** Currently, this facility is not ready for use in a manufacturing capacity.

Item 3. Legal Proceedings.

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. Plaintiffs consolidated amended complaint, filed on July 21, 2014, seeks to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleges that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seeks damages in an unspecified amount.

Table of Contents

Pursuant to the court s June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, which remains pending. In addition, another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Chris Garabedian, and Sandy Mahatme, 1:14-cv-14318, asserting violations of Section 10(b) of the Exchange Act and SEC Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme. Plaintiff alleges that the defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company s data for submission of an new drug application for eteplirsen and the likelihood of the FDA accepting a new drug application based on that data. Plaintiff seeks compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta will move to dismiss the complaint. Additionally, on September 23, 2014, a derivative suit was filed against the Company s Board of Directors with the Court of Chancery of the State of Delaware (Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157). The claims allege, among other things, that (i) the Company s non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company s CEO was also excessive and such fees were the basis for the CEO not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, are disgorgement and rescindment of excessive or unfair payments and equity grants to the CEO and directors, unspecified damages plus interest, a class action declaration for the suit, declaring approval of the Company s Amended and Restated 2011 Equity Plan at the 2013 meeting ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff s attorney fees.

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

Our Common Stock is quoted on The NASDAQ Global Select Market under the symbol SRPT. Prior to January 2, 2014, our Common Stock was quoted on The NASDAQ Global Market. The following table sets forth the high and low intraday sales prices as reported by The NASDAQ Global Select 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, 2014		
First Quarter	$ 31.28	$ 17.50
Second Quarter	$ 40.00	$ 20.89
Third Quarter	$ 31.35	$ 18.59
Fourth Quarter	$ 24.95	$ 12.58
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 23, 2015, we had 193 stockholders of record of our common stock.

Dividends

We did not declare or pay cash dividends on our common stock in 2014, 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, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology Index. This graph assumes an investment of $100 on December 31, 2009 in each of our common stock, the NASDAQ Composite Index, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology 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 U.S. Securities and Exchange Commission 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.

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 consolidated 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 Operations,* and *Item 8, Financial Statements and Supplementary Data*.

	For the Year Ended December 31,				
	2014	2013	2012	2011	2010
	(in thousands, except per share amounts)				
Operations data:					
Revenue	$ 9,757	$ 14,219	$ 37,329	$ 46,990	$ 29,420
Research and development	94,231	72,909	52,402	66,862	35,972
General and administrative	49,315	31,594	14,630	16,055	14,382
Operating loss	(133,789)	(90,284)	(29,703)	(35,927)	(20,934)
Interest income and other, net	779	326	354	587	259
(Loss) gain on change in warrant valuation	(2,779)	(22,027)	(91,938)	33,022	(11,502)
Net loss	$ (135,789)	$ (111,985)	$ (121,287)	$ (2,318)	$ (32,177)
Net loss per share basic and diluted	$ (3.39)	$ (3.31)	$ (5.14)	$ (0.11)	$ (1.74)
Balance sheet data:					
Cash and cash equivalents	$ 73,551	$ 256,965	$ 187,661	$ 39,904	$ 33,589
Working capital	210,929	234,840	115,022	24,583	(8,019)
Total assets	295,033	291,569	204,993	54,368	45,976
Stockholders equity (deficit)	247,653	247,192	123,679	31,017	(2,817)

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-targeted therapeutics for the treatment of rare, infectious and other 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-targeted 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 DMD product candidate, eteplirsen, designed to skip exon 51. We are also developing therapeutics using our technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD in the U.S. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe but unmet medical need. We are in the process of conducting or starting several studies for product candidates designed to skip exons 45, 51 and 53 in the U.S. and in Europe. These include an ongoing open label extension study following completion of our initial Phase IIb clinical trials, several clinical trials in exon 51 amenable genotypes, including a confirmatory study in ambulatory patients, studies on participants with early stage and advanced stage DMD and a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53. Additionally, we have begun Phase I of a Phase I/IIa clinical trial for an exon 53 skipping product candidate in the European Union (E.U.) with the SKIP-NMD Consortium. We plan to file a new drug application (NDA) for eteplirsen for the treatment of DMD by mid-year 2015, although we will continue to evaluate our NDA submission plans based on discussions with the Food and Drug Administration (FDA) and as additional data become available. We have also leveraged the capabilities of our RNA-targeted technology platforms to develop therapeutics for the treatment of infectious diseases such as influenza, Marburg and Ebola under prior contracts with the Department of Defense (DoD), however, further development of these product candidates would be conditioned, in part, on obtaining additional funding, collaborations or emergency use. Our discovery and research programs include collaborations with various third parties and focus on developing therapeutics in rare, genetic, anti-infective, neuromuscular and central nervous system diseases amongst other diseases. We are exploring the application of our proprietary phosphorodiamidate morpholino oligomer (PMO) platform technology and toll-like receptor (TLR) technology in various diseases including drug resistant bacteria, DMD, Becker muscular dystrophy (Becker), Progeria, Adult Onset Pompe Disease, Lupus and Graft-versus-Host Disease.

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. 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

Table of Contents

and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support our product candidates.

The basis of our novel RNA-targeted therapeutics is the PMO. Our next generation PMO-based chemistries include PMO-X®, PMO*plus*® and PPMO. PMOs are highly resistant to degradation by enzymes, potentially enabling robust and sustained biological activity. In contrast to other RNA-targeted therapeutics, which are usually designed to down-regulate protein expression, our technologies are designed to selectively up-regulate or down-regulate protein expression, and more importantly, create novel proteins. PMOs have demonstrated inhibition of messenger RNA (mRNA) translation and alteration of pre-mRNA splicing. The chemistry of PMO-based molecules has the potential to reduce off-target effects, such as the immune stimulation often observed with ribose-based RNA technologies. We believe that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies. In addition, PMOs are highly adaptable molecules: with minor structural modifications, they can potentially be rapidly designed to target specific tissues, genetic sequences, or pathogens, and therefore, we believe they could potentially be applied to treat a broad spectrum of diseases.

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

As of December 31, 2014, we had approximately $211.1 million of cash, cash equivalents and investments, consisting of $73.6 million of cash and cash equivalents, $136.8 million of short-term investments and $0.8 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months. As of December 31, 2014, we have completed all development activities under the agreements with the DoD. We are currently exploring possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and influenza product candidates. Without funding from the U.S. government, we would likely curtail certain infectious disease research and development efforts, though we may pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations with or license our technology to other companies.

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

Summary and Timeline of Eteplirsen Data Disclosure

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

Table of Contents

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 observed, 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 (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 the measurement taken of 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 the measurement taken of 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.

All participants in Study 201 were enrolled in an open-label extension study 4658-US-202 (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 (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).

In July 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 (ITT) 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

Table of Contents

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.

In October 2012, we announced 48-week results from Study 202 which indicated that treatment with eteplirsen met the predefined primary efficacy endpoint, increase in the measurement taken of 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 the measurement taken of 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 the measurement taken of dystrophin-positive fibers to 38.3% of normal ($p<0.009$).

In the predefined analysis of the study s ITT 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.

In December 2012, we announced updated data from Study 202 which showed patients treated with eteplirsen and evaluable on ambulatory measures in modified intent-to-treat population (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, 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

Table of Contents

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 based on the measurements taken, 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.

In April 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 13.4 meters, or 5 percent decline 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-treated 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.

In June 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 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.

In September 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 17.5 meters, or 5 percent decline 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,

Table of Contents

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.

In January 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 (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, in February 2014, we announced that results through more than two years of treatment showed stable pulmonary function in the 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.

In July 2014, we announced that at 144 weeks, patients in the 30 mg/kg and 50 mg/kg eteplirsen cohorts who were able to perform the 6MWT (mITT population; n=6) experienced a decline of 33.2 meters, or about 8.5 percent, from baseline in walking ability. A statistically significant treatment benefit of 75.1 meters ($p \leq 0.004$) was observed for the mITT population compared with the placebo/delayed-treatment cohort (n=4), which initiated treatment at Week 25 following 24 weeks of placebo. After experiencing a substantial decline of 68.4 meters from baseline to Week 36, the placebo/delayed-treatment cohort demonstrated a decline of 39.0 meters in walking ability from Week 36 through Week 144, the period from which meaningful levels of dystrophin were likely produced. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days. Respiratory muscle function from baseline through Week 144 in the ITT population (n=12), as measured by maximum inspiratory and expiratory pressure (MIP and MEP), showed a 14.7 percent mean increase in MIP and a 12.8 percent mean increase in MEP. Analyses of MIP percent predicted (MIP adjusted for weight) and MEP percent predicted (MEP adjusted for age) demonstrated a mean change from 91.7 percent at baseline to 93.9 percent at Week 144 in MIP percent predicted, and a mean change from 79.3 percent at baseline to 75.7 percent at Week 144 in MEP percent predicted. In addition, there was a mean increase in forced vital capacity (FVC), a measure of lung volume, of 11.0 percent. FVC percent predicted (FVC adjusted for age and height) was maintained above a mean of 90 percent at Week 144, with 101.3 percent at baseline and 90.9 percent at Week 144. Through 144 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.

In January 2015, we announced that at Week 168, the six patients in the mITT population in the 30 and 50 mg/kg eteplirsen cohorts who were able to perform the 6MWT experienced a decline of 76.7 meters, or about 19.5 percent, from baseline in walking ability. A statistically significant treatment benefit of 65.4 meters ($p \leq 0.017$) was observed compared with the placebo/delayed-treatment cohort (n=4), which initiated treatment at Week 25 following 24 weeks of placebo. This cohort, after experiencing a substantial decline of 68.4 meters from baseline to Week 36, demonstrated a decline of 73 meters in walking ability from Week 36 through Week 168, the period from which meaningful levels of dystrophin were likely produced. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days. Respiratory muscle function from baseline through Week 168 in the Intent-to-Treat population (n=12), as measured by maximum inspiratory and expiratory pressure (MIP and MEP), continued to show a 11.1 percent mean increase in MIP and a 14.7 percent mean increase in MEP. Analyses of MIP percent predicted (MIP adjusted for weight) and MEP percent predicted (MEP adjusted for age) demonstrated a mean change from 91.7 percent at baseline to 89.5 percent at Week 168 in MIP percent predicted, and a mean change from 79.3 percent at baseline to 74.3 percent at Week 168 in MEP percent predicted. In addition, there was a mean increase in FVC, a measure of lung volume, of 11.6 percent. FVC percent predicted (FVC adjusted for age and height) was maintained above a mean of 90 percent at Week 168, with 101.3 percent at Baseline and 91.9 percent at Week 168. Through 168 weeks,

Table of Contents

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.

Summary and Timeline of Marburg Product Candidate Data Disclosures

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 (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, one of the two components that make up AVI-6003, 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. 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 1-, 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 and 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 (JMP-MCS) (renamed Medical Countermeasure Systems in 2013) of the DoD, further development of AVI-7288 is planned pursuant to FDA s Animal Efficacy Rule.

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, 2014, we had completed all development activities of our contracts with the DoD.

Table of Contents

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

	For the Year Ended December 31		
	2014	2013	2012
		(in thousands)	
July 2010 Agreement (*Ebola and Marburg Intravenous Administration)*	$ 6,816	$ 9,064	$ 36,557
June 2010 Agreement (*H1N1/Influenza*)		427	
August 2012 Agreement (*Intramuscular Administration*)		2,791	673
European Union SKIP-NMD Agreement (*DMD*)	1,432	1,263	
Children s National Medical Center Agreement (*DMD*)	659	674	
Carolinas Medical Center Agreement (*DMD*)	850		
Other Agreements			99
Total	$ 9,757	$ 14,219	$ 37,329

July 2010 Agreement (Ebola and Marburg Intravenous Administration)

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

The Marburg portion of the contract was structured into four segments and had an aggregate remaining period of performance spanning approximately four years if the DoD exercised its options for all segments. Activities under the first segment began in July 2010 and included preclinical studies and Phase I studies in healthy volunteers. In February 2014, we announced positive safety results from the Phase I multiple ascending dose study of AVI-7288. The remaining Marburg portion of the contract expired in July 2014. For the years ended December 31, 2014, 2013 and 2012, we recognized $6.8 million, $9.1 million and $36.6 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of December 31, 2014 and only revenue for contract finalization, if any, is expected in the future.

June 2010 Agreement (H1N1/Influenza)

In June 2010, we entered into an agreement 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 (TMT) program of the DoD. The period of performance for this agreement ended in June 2011. We recognized $0.4 million associated with this agreement for the year ended December 31, 2013, which was the result of an indirect rate adjustment.

August 2012 Agreement (Intramuscular Administration)

In August 2012, we were awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, candidate for treatment of the Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. Accordingly, no revenue was recognized since the conclusion of the contract. For the years ended December 31, 2013 and 2012, we recognized $2.8 million and $0.7 million, respectively, as revenue under this agreement.

Table of Contents

European Union SKIP-NMD Agreement (DMD)

In November 2012, we entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic. For the years ended December 31, 2014 and 2013, we recognized $1.4 million and $1.3 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of December 31, 2014 and only revenue for contract finalization, if any, is expected in the future.

Children s National Medical Center Agreement (DMD)

In July 2013, we entered into an agreement totaling $1.3 million to provide drug product to Children s National Medical Center (CNMC) to conduct research related to our DMD program. For each of the years ended December 31, 2014 and 2013, we recognized $0.7 million as revenue under the agreement. The period of performance for this contract concluded in March 2014 and no revenue under this contract is expected in the future.

Carolinas Medical Center Agreement (DMD)

We entered into a collaboration agreement with Carolinas Medical Center (CMC) to co-develop one of our DMD programs. Under the agreement, CMC was obligated to reimburse certain preclinical costs incurred by us. All preclinical work was completed and we recognized revenue of $0.9 million for the year ended December 31, 2014.

Key Financial Metrics

Revenue

Government Research Contract and Grant Revenue. 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, 2014, we had completed all development activities of our contracts with the U.S. government. 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 on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments.

We defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee that is separate and independent of our performance under the other elements of the arrangement. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2014, we had deferred revenue of $3.3 million, which primarily represents up-front fees which we will recognize as revenue upon settlement of certain obligation contingencies.

Expenses

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

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

Table of Contents

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

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

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

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

Loss on Change in Warrant Valuation. Warrants issued in connection with our January and August 2009 financings were classified as liabilities as opposed to equity due to their settlement terms. These warrants were non-cash liabilities and we were not required to expend any cash to settle these liabilities. The fair value of these warrants was recorded on our consolidated balance sheets at the date of issuance and the warrants were marked to market at each financial reporting period, with changes in the fair value recorded as Loss on change in warrant valuation in our consolidated statements of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates related to the inputs used in the model and can result in significant swings in the fair value primarily due to changes in our stock price. As of December 31, 2014, there were no outstanding warrants as all warrants issued in January and August 2009 were exercised or expired. For more information, please read *Note 9, Warrants* to 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 (U.S. 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

Table of Contents

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;
- income tax; and
- accounting for and valuation of liability classified warrants.

Revenue Recognition

We have historically generated revenue from our U.S. government research contracts and other grants. During the periods presented, substantially all of our revenue was 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 on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments.

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.

We defer and capitalize non-refundable advance payments for goods or services that will be used or rendered for future research and development activities pursuant to an executory contractual arrangement and recognized as an expense as the related goods are delivered or the related services are performed. If we do not expect the goods to be delivered or services to be rendered, the advance payment capitalized will be charged as an expense.

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.

Stock Compensation Expense

To determine stock-based compensation expense, we apply the provisions of Financial Accounting Standards Board (FASB), Accounting Standards Codification (ASC), Topic 718, Share-Based Payments. We use the Black-Scholes-Merton option-pricing model to determine the fair value of stock-based awards on the date of grant. The Black-Scholes-Merton option-pricing model requires the use of subjective and complex

assumptions which include the award s expected term and the price volatility of the underlying stock. We

Table of Contents

recognize the fair value of the portion of the awards expected to vest as expense over the requisite vesting periods on a straight-line basis for the entire award. Stock awards granted to employees vest over a four-year period and have a ten-year term. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

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. Please read *Note 13, Stock-Based Compensation* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of stock-based compensation.

Income Tax

We follow 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 our intention to reinvest the earnings of our non-U.S. subsidiaries in those operations and not to repatriate the earnings to the U.S. Accordingly, we do 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, we have not had any earnings in our 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. We recognize the effect of income tax positions only if those positions are more likely than not to be sustained upon an examination. Please read *Note 16, Income Tax* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of income tax.

Warrant Liability

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 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. As of December 31, 2014, all warrants were exercised or expired. Please read *Note 9, Warrants* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of warrants.

Table of Contents

Results of Operations for the years ended December 31, 2014, 2013 and 2012

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

	For the Year Ended December 31,			% Change	
	2014	2013	2012	2014 vs. 2013	2013 vs. 2012
	(in thousands, except per share amounts)				
Revenue	$ 9,757	$ 14,219	$ 37,329	(31)%	(62)%
Operating expenses:					
Research and development	94,231	72,909	52,402	29%	39%
General and administrative	49,315	31,594	14,630	56%	116%
Total operating expenses	143,546	104,503	67,032	37%	56%
Operating loss	(133,789)	(90,284)	(29,703)	48%	204%
Other income (loss):					
Interest income and other, net	779	326	354	139%	(8)%
Loss on change in warrant valuation	(2,779)	(22,027)	(91,938)	(87)%	(76)%
Net loss	$ (135,789)	$ (111,985)	$ (121,287)	21%	(8)%
Net loss per share basic and diluted	$ (3.39)	$ (3.31)	$ (5.14)	2%	(36)%

Revenue

Revenue for 2014 decreased by $4.5 million, or 31%, compared to 2013. The decrease was due to a decrease of $2.8 million in the August 2012 Agreement which was concluded in the third quarter of 2013, $2.2 million in revenue associated with the Marburg portion of the July 2010 Agreement which expired in July 2014 and $0.4 million in the June 2010 Agreement. These decreases were partially offset by an increase of $0.9 million in the CMC Agreement and $0.2 million in the E.U. SKIP-NMD Agreement.

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 the July 2010 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 August 2012 Agreement of $2.8 million in 2013 as compared to $0.7 million in 2012. Additionally, there were revenues from the E.U. SKIP Agreement and the CNMC Agreement of $1.3 million and $0.7 million, respectively, which did not have any revenue associated with them in 2012.

Research and Development Expenses

Research and development expenses for 2014 increased by $21.3 million, or 29%, compared to 2013. The increase primarily includes $10.8 million in personnel costs which include $4.4 million increase in stock-based compensation expense, $3.9 million in clinical and pre-clinical expenses, $3.1 million in professional services, $3.0 million in facility-related expenses and $1.6 million in general research and drug discovery efforts. The increase was partially offset by a decrease of $2.3 million in manufacturing 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.

Table of Contents

General and Administrative Expenses

General and administrative expenses for 2014 increased by $17.7 million, or 56%, compared to 2013. The increase in general and administrative expenses is primarily due to increases of $7.9 million in professional services, $6.1 million in personnel costs including $4.8 million stock-based compensation expenses and facility-related expenses of $3.1 million.

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.

Interest Income and Other, Net

Interest income and other, net for 2014 increased by 0.5 million, or 139%. The increase was primarily driven by interest income from short-term investments.

Interest income and other, net, for 2013 remained consistent compared to 2012.

Loss on Change in Warrant Valuation

Loss on change in warrant valuation in 2014 decreased by $19.2 million, or 87% compared to 2013. Loss on change in warrant valuation in 2013 decreased by $69.9 million, or 76% compared to 2012. For both periods, the decrease was primarily attributable to the change in our stock price and decrease in the number of outstanding warrants. Please read *Note 9, Warrants* of the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Net Loss

Net loss for 2014 increased by $23.8 million, or 21%, compared to 2013. The increase in net loss was primarily due to increases of $21.3 million in research and development expenses and $17.7 million in general and administrative expenses due to corporate growth as well as a decrease of $4.5 million in revenue. This was partially offset by a decrease of $19.2 million in loss on change of warrant valuation.

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

Liquidity and Capital Resources

The following table summarizes our financial condition for each of the periods indicated:

	For the Year Ended December 31,			% Change	
	2014	2013	2012	2014 vs. 2013	2013 vs. 2012
	(in thousands)				
Financial assets:					
Cash and cash equivalents	$ 73,551	$ 256,965	$ 187,661	(71)%	37%
Short-term investments	136,793			NA	NA
Restricted cash and investments	782	7,897		(90)%	NA
Total cash, cash equivalents and investments	$ 211,126	$ 264,862	$ 187,661	(20)%	41%
Borrowings:					
Long-term debt	$ 1,574	$ 1,668	$ 1,757	(6)%	(5)%
Notes payable	4,754			NA	NA
Total borrowings	$ 6,328	$ 1,668	$ 1,757	279%	(5)%
Working capital:					
Current assets	$ 247,796	$ 270,806	$ 193,908	(8)%	40%
Current liabilities	36,867	35,966	78,886	3%	(54)%
Total working capital	$ 210,929	$ 234,840	$ 115,022	(10)%	104%

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

Our primary source of revenue has historically been from development of product candidates pursuant to government contracts and other grants. U.S. government funding is subject to the government s appropriations process and the government has the right to terminate such contracts for convenience. As of December 31, 2014, we have completed all development activities under the agreements with the DoD. We are currently exploring possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and Influenza product candidates; however, if we do not succeed in these efforts, we will likely curtail their further development. Currently, we do not generate any revenue from the commercial sale of our product candidates.

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

- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;
- the timing and costs associated with our clinical trials and preclinical studies;
- the timing and costs associated with commercialization of eteplirsen should marketing approval ever be granted; and

the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technology. We cannot provide

Table of Contents

assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

The following table summarizes our cash flow activity for each of the periods indicated:

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Cash provided by (used in):			
Operating activities	$ (128,539)	$ (64,695)	$ (29,694)
Investing activities	(159,030)	(11,672)	(1,145)
Financing activities	104,155	145,671	178,596
(Decrease) increase in cash and cash equivalents	$ (183,414)	$ 69,304	$ 147,757

Operating Activities.

The increase in the amount of cash used in operating activities of $63.8 million for 2014 compared to 2013 was primarily due to an increase in operating loss of $43.5 million driven by lower government contract revenue and higher research and development and general and administrative expenses due to corporate growth and $34.4 million from unfavorable changes in operating assets and liabilities primarily driven by advanced payments related to our future inventory commitments. This was partially offset by an increase of $13.6 million in non-cash adjustments to net loss.

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 an increase of $8.0 million in non-cash adjustments to net loss in 2013 as compared to 2012 as well as $17.6 million from favorable changes in operating assets and liabilities.

Investing Activities.

The increase in the amount of cash used in investing activities of $147.4 million for 2014 compared to 2013 was primarily due to the purchase of available-for-sale securities of $274.4 million and an increase of $23.1 million in capital expenditures partially due to the acquisition of the Andover facility. The increase was partially offset by maturity of available-for-sale securities of $134.9 million and restricted investments of $7.3 million which were purchased in 2013.

The increase in the amount of cash used in investing activities of $10.5 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.

Financing Activities.

Cash inflows from financing activities in 2014 decreased by $41.5 million compared to 2013. In April 2014, we sold approximately 2.7 million shares of common stock at an offering price of $38.00 per share. After

Table of Contents

deducting the underwriting discounts and offering related transaction costs, we received aggregate net proceeds of approximately $94.5 million, $30.6 million lower than prior year equity financings. Additionally, there were decreases of $10.8 million from warrant exercises and $1.7 million from option exercises. These were partially offset by $1.0 million from the employee stock purchase program.

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 $18.6 million in net proceeds from warrant exercises and $2.5 million from stock option exercises during 2013 for which we issued approximately 2.6 million shares of additional common stock.

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.

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, 2014:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
			(in thousands)		
Long-term debt(1)	$ 2,079	$ 171	$ 343	$ 343	$ 1,222
Notes payable(1)	5,019	2,516	2,503		
Operating leases	29,163	4,382	9,341	9,812	5,628
Purchase obligations (2)	140,325	56,232	64,128	19,965	
Total	$ 176,586	$ 63,301	$ 76,315	$ 30,120	$ 6,850

(1) Interest is included.

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

(3) We are obligated to make up to $35.8 million of future development and commercial milestone payments associated with some of our collaboration and license agreements.

Recent Accounting Pronouncements

Please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

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

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, government and government agency bonds and high-grade corporate bonds with maturities of twenty-four months or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of December 31, 2014, we had $211.1 million of cash, cash equivalents and investments, comprised of $73.6 million of cash and cash equivalents, $136.8 million short-term investments and

Table of Contents

$0.8 million of restricted cash and investments. 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. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. For each of the years ended December 31, 2014 and 2013, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1 million to our interest rate sensitive instruments.

Item 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.

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

None.

Item 9A. Controls and Procedures.

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 are effective to 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.

Internal Control over Financial Reporting

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;

Table of Contents

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.

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. Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2014. In making this assessment, management used the criteria in Internal Control Integrated Framework (1992) (the 1992 framework) issued by the Committee of Sponsoring Organizations of the Treadway Commission. In May 2013, COSO issued an updated Internal Control-Integrated Framework (the 2013 framework). Management continued to apply the 1992 framework in its 2014 assessment of internal controls and expects to adopt the 2013 framework during fiscal year 2015.

Based on this assessment, management has concluded that, as of December 31, 2014, our internal control over financial reporting was effective. The effectiveness of our internal control over financial reporting as of December 31, 2014 has been audited by KPMG LLP, an independent registered public accounting firm, as stated in their report which appears in this Annual Report on Form 10-K.

Changes in Internal Control over Financial Reporting

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 quarter ended December 31, 2014 that our certifying officers concluded materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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 internal control over financial reporting as of December 31, 2014, 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. 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.

In our opinion, Sarepta Therapeutics, Inc. and subsidiaries maintained, in all material respects, effective internal control over financial reporting as of December 31, 2014, based on criteria established in Internal Control Integrated Framework (1992) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

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 as of December 31, 2014 and 2013, and the related consolidated statements of operations and comprehensive loss, stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2014, and our report dated February 26, 2015 expressed an unqualified opinion on those consolidated financial statements.

(signed) KPMG LLP

Cambridge, Massachusetts

February 26, 2015

Table of Contents

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 2015 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 2015 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 2015 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 2015 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 2015 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:

(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 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	Amended and Restated Bylaws.	8-K	001-14895	3.1	9/25/14	
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-K	001-14895	10.1	6/16/11	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
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, 2013 by and between Sarepta Therapeutics, Inc. and Sandesh Mahatme.	10-K	001-14895	10.24	3/3/14	
10.25	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and David Tyronne Howton.	10-K	001-14895	10.25	3/3/14	
10.26	Executive Inducement Stock Option Agreement between Arthur Krieg and Sarepta Therapeutics, Inc.	10-K	001-14895	10.26	3/3/14	
10.27	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan.	10-K	001-14895	10.27	3/3/14	
10.28	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan	10-K	001-14895	10.28	3/3/14	
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	

Table of Contents

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.30*	Amended and Restated Exclusive License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc. 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	
10.56	Purchase and Sale Agreement dated May 22, 2014 between Sarepta Therapeutics, Inc. and Eisai Inc.	10-Q	001-14895	10.1	8/7/14	
10.57	Offer Letter dated January 6, 2014 by and between Sarepta Therapeutics, Inc. and Arthur Krieg, M.D.	10-Q	001-14895	10.1	5/8/14	
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.

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: February 26, 2015

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 February 26, 2015:

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/ John C. Hodgman John C. Hodgman	Interim Chairman of the Board
/s/ M. Kathleen Behrens M. Kathleen Behrens, Ph.D.	Director
/s/ Anthony Chase Anthony Chase	Director
/s/ William Goolsbee William Goolsbee	Director

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

Table of Contents

SAREPTA THERAPEUTICS, INC.

CONSOLIDATED FINANCIAL STATEMENTS

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 as of December 31, 2014 and 2013, and the related consolidated statements of operations and comprehensive loss, stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2014. 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.

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 as of December 31, 2014 and 2013, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2014, 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. and subsidiaries internal control over financial reporting as of December 31, 2014, 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 February 26, 2015 expressed an unqualified opinion on the effectiveness of the Company s internal control over financial reporting.

(signed) KPMG LLP

Cambridge, Massachusetts

February 26, 2015

Table of Contents

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(in thousands, except share data)

	As of December 31, 2014	As of December 31, 2013
Assets		
Current Assets:		
Cash and cash equivalents	$ 73,551	$ 256,965
Short-term investments	136,793	
Accounts receivable	2,416	3,530
Restricted investments		7,250
Other current assets	35,036	3,061
Total Current Assets	247,796	270,806
Restricted cash and investments	782	647
Property and equipment, net	38,501	15,049
Patent costs, net of accumulated amortization of $2,081 and $1,622 as of December 31, 2014 and 2013, respectively	5,891	5,042
Other assets	2,063	25
Total Assets	$ 295,033	$ 291,569
Liabilities and Stockholders Equity		
Current Liabilities:		
Accounts payable	$ 12,408	$ 8,080
Accrued expenses	17,366	14,601
Current portion of long-term debt	98	92
Current portion of notes payable	2,492	
Warrant liability		9,006
Deferred revenue	3,318	3,299
Other liabilities	1,185	888
Total Current Liabilities	36,867	35,966
Long-term debt	1,476	1,576
Notes payable	2,262	
Deferred rent	6,775	6,835
Total Liabilities	47,380	44,377
Commitments and contingencies (Note 18)		
Stockholders Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding		
Common stock, $.0001 par value, 50,000,000 shares authorized; 41,311,512 and 37,751,920 issued and outstanding as of December 31, 2014 and 2013, respectively	4	4
Additional paid-in capital	926,769	790,424
Accumulated other comprehensive loss	(95)	
Accumulated deficit	(679,025)	(543,236)

Total Stockholders Equity	247,653	247,192
Total Liabilities and Stockholders Equity	$ 295,033	$ 291,569

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except per share data)

	For the Year Ended December 31,		
	2014	2013	2012
Revenue from research contracts and other grants	$ 9,757	$ 14,219	$ 37,329
Operating expenses:			
Research and development	94,231	72,909	52,402
General and administrative	49,315	31,594	14,630
Total operating expenses	143,546	104,503	67,032
Operating loss	(133,789)	(90,284)	(29,703)
Other income (loss):			
Interest income and other, net	779	326	354
Loss on change in warrant valuation	(2,779)	(22,027)	(91,938)
Total other loss	(2,000)	(21,701)	(91,584)
Net loss	$ (135,789)	$ (111,985)	$ (121,287)
Other comprehensive loss:			
Unrealized loss on short-term securities available-for-sale	(95)		
Total other comprehensive loss	(95)		
Comprehensive loss	$ (135,884)	$ (111,985)	$ (121,287)
Net loss per share basic and diluted	$ (3.39)	$ (3.31)	$ (5.14)
Weighted average number of common stock outstanding for computing basic and diluted net loss per share	40,026	33,850	23,602

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

Consolidated Statements of Stockholders Equity

(in thousands)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders Equity
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	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
Vest of restricted stock units	31					
Shares withheld for taxes	(7)		(226)			(226)
Grant of restricted stock awards	6					
Issuance of common stock for cash, net of offering costs	3,441	1	125,103			125,104
Stock-based compensation			11,127			11,127
Net loss					(111,985)	(111,985)
BALANCE AT DECEMBER 31, 2013	**37,752**	**4**	**790,424**		**$ (543,236)**	**$ 247,192**
Exercise of options for common stock	86		980			980
Exercise of warrants for common stock	766		19,536			19,536
Vest of restricted stock units	7					
Shares withheld for taxes	(1)		(34)			(34)
Grant of restricted stock awards	6					
Issuance of common stock for cash, net of offering costs	2,650		94,503			94,503
Issuance of common stock under employee stock purchase plan	46		1,015			1,015
Stock-based compensation			20,345			20,345
Unrealized loss from available-for-sale securities				(95)		(95)
Net loss					(135,789)	(135,789)
BALANCE AT DECEMBER 31, 2014	**41,312**	**$ 4**	**$ 926,769**	**$ (95)**	**$ (679,025)**	**$ 247,653**

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

Consolidated Statements of Cash Flows

(in thousands)

	For the Year Ended December 31		
	2014	2013	2012
Cash flows from operating activities:			
Net loss	$ (135,789)	$ (111,985)	$ (121,287)
Adjustments to reconcile net loss to net cash flows used in operating activities:			
Depreciation and amortization	3,690	1,277	1,525
Amortization of premium on available-for-sale securities	2,432		
Loss on abandonment of patents and disposal of property and equipment	128	590	357
Stock-based compensation	20,345	11,127	3,078
Loss on change in warrant valuation	2,779	22,027	91,938
Non-cash interest expense	12		
Changes in operating assets and liabilities, net:			
Net decrease (increase) in accounts receivable	1,114	1,183	(1,080)
Net (increase) decrease in other assets	(34,013)	1,618	(2,507)
Net increase (decrease) in accounts payable, accrued expenses and other liabilities	10,763	9,468	(1,718)
Net cash used in operating activities	(128,539)	(64,695)	(29,694)
Cash flows from investing activities:			
Purchase of restricted investments		(7,897)	
Purchase of property and equipment	(25,444)	(2,370)	(108)
Patent costs	(1,381)	(1,405)	(1,037)
Purchase of available-for-sale securities	(274,368)		
Maturity of available-for-sale securities	134,913		
Release and maturity of restricted investments	7,250		
Net cash used in investing activities	(159,030)	(11,672)	(1,145)
Cash flows from financing activities:			
Proceeds from exercise of options and warrants and the sale of common stock, net of offering costs	104,249	145,986	178,681
Repayments of long-term debt	(94)	(89)	(85)
Other financing activities, net		(226)	
Net cash provided by financing activities	104,155	145,671	178,596
(Decrease) increase in cash and cash equivalents	(183,414)	69,304	147,757
Cash and cash equivalents:			
Beginning of period	256,965	187,661	39,904
End of period	$ 73,551	$ 256,965	$ 187,661
Supplemental disclosure of cash flow information:			
Cash paid during the year for interest	$ 77	$ 144	$ 86
Supplemental schedule of noncash investing activities and financing activities:			
Issuance of common stock in satisfaction of warrants and other liabilities	$ 11,785	$ 78,214	$ 32,191
Issuance of notes payable in relation to the purchase of certain real and personal property located in Andover, Massachusetts	$ 4,613	$	$
Capitalized interest	$ 137	$	$

Tenant improvements paid by landlord	$ 153	$ 6,214	$
Property and equipment included in accrued expenses	$ 277	$ 3,964	$
Patent costs included in accrued expenses	$ 270	$ 195	$

See accompanying notes to consolidated financial statements.

Table of Contents

Sarepta Therapeutics, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. ORGANIZATION AND NATURE OF 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-targeted therapeutics for the treatment of rare, infectious and other diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. The Company is also developing therapeutics using its technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

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

As of December 31, 2014, the Company had approximately $211.1 million of cash, cash equivalents and investments, consisting of $73.6 million of cash and cash equivalents, $136.8 million of short-term investments and $0.8 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments is sufficient to fund its current operational plan for the next twelve months. Without funding from the U.S. government, the Company would likely curtail certain infectious disease research and development efforts, though it may pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

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

Estimates and Uncertainties

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

Reclassifications

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

Table of Contents

Additionally, the Company has revised the presentation as well as the caption of certain cash flows from operating activities within the consolidated statements of cash flows to conform to the current period presentation. Net decrease in other assets of $1.6 million for the year ended December 31, 2013 is broken out from Net increase in accounts receivable and other assets and presented gross on the consolidated statements of cash flows. This revision had no impact on net cash used in operating activities or change in cash and cash equivalents.

Fair Value Measurements

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

Level 1 quoted prices for identical instruments in active markets;

Level 2 quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and

Level 3 valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The fair value of most of the Company s financial assets is categorized as Level 2 within the fair value hierarchy. These financial assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing market observable data. For additional information related to fair value measurements, please read *Note 4, Fair Value Measurements* to the consolidated financial statements.

Cash and Cash Equivalents

Only investments that are highly liquid and readily convertible to cash and have original maturities of three months or less are considered cash equivalents. As of December 31, 2014 and 2013, cash equivalents were comprised of money market funds.

Available-For-Sale Debt Securities

Available-for-sale debt securities are recorded at fair market value and unrealized gains and losses are included in accumulated other comprehensive loss in stockholder s equity. Realized gains and losses are reported in interest income and other, net, on a specific identification basis.

Accounts Receivable

The Company s accounts receivable primarily arise from government research contracts and other grants. They are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily from government agencies and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. The balance for unbilled receivables for both years ended December 31, 2014 and 2013 was $2.4 million, all of which is subject to government audit and will not be collected until the completion of the audit.

Property and Equipment

Property and equipment are initially recorded at cost, including the acquisition cost and all costs necessarily incurred to bring the asset to the location and working condition necessary for its intended use. The cost of normal, recurring, or periodic repairs and maintenance activities related to property and equipment are expensed

Table of Contents

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. Interest costs incurred during the construction period of major capital projects are capitalized until the asset is ready for its intended use, at which point the interest costs are amortized as depreciation expense over the life of the underlying asset.

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
Software and computer equipment	5 years
Leasehold improvements	Lesser of the useful life or the term of the respective lease
Land	Not depreciated
Building	30 years
Construction in Progress	Not depreciated until put into service

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 $0.5 million, $0.4 million and $0.6 million for the years ended December 31, 2014, 2013 and 2012, respectively. The Company also expensed the remaining net book value of previously capitalized patents that were later abandoned of $0.1 million, $0.5 million and $0.4 million in 2014, 2013 and 2012, respectively, which were included in research and development expenses on the consolidated statements of operations and comprehensive loss.

The following table summarizes the estimated future amortization for patent costs for the next five years:

	As of December 31, 2014 (in thousands)
2015	$ 479
2016	479
2017	464
2018	456
2019	450
Total	$ 2,328

Impairment of Long-Lived Assets

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

Table of Contents

Revenue Recognition

All of the Company s revenue is generated from government research contracts and other grants. 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 government research contracts during the period in which the related expenses are incurred and presents such revenues and related expenses on a gross basis in the consolidated financial statements. The Company s government contracts are subject to government audits, which may result in catch-up adjustments. For additional information related to revenue, please read *Note 12, Government Contracts* to the consolidated financial statements.

The Company defers recognition of non-refundable up-front fees if it has 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 its 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 or because the services can only be performed by it, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2014, the Company had deferred revenue of $3.3 million, which primarily represents up-front fees which it will recognize as revenue upon settlement of certain obligation contingencies.

Research and Development

Research and development expenses consist of costs associated with research activities as well as those with the Company s product development efforts, conducting preclinical studies, clinical trials and manufacturing activities. Research and development expenses are expensed as incurred. Payments made for research and development services prior to the services being rendered are recorded as prepaid assets on the Company s consolidated balance sheets and are expensed as the services are provided.

Nonrefundable advance payments for goods or services that will be used or rendered for future research and development activities pursuant to an executory contractual arrangement will be deferred and capitalized, and recognized as an expense as the related goods are delivered or the related services are performed. If the Company does not expect the goods to be delivered or services to be rendered, the advance payment capitalized will be charged to expense.

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 external services, such as data management and statistical analysis support and materials and supplies used in support of clinical programs. Indirect costs of the Company s clinical programs include salaries, stock-based compensation and an allocation of our 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-Based Compensation

The Company s stock-based compensation programs include stock options, restricted stock awards (RSAs), restricted stock units (RSUs), stock appreciation rights (SARs) and employee stock purchase program (ESPP). The Company accounts for stock-based compensation using the fair value method.

Table of Contents

The fair values of stock options and SARs are estimated on the date of grant using the Black-Scholes-Merton option-pricing model. The fair values of RSAs and RSUs are based on the fair market value of the Company s common stock on the date of the grant. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the vesting period of the grants. For stock awards with performance-vesting conditions, the Company does not recognize compensation expense until it is probable that the performance-vesting condition will be achieved.

Under the Company s ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company s common stock on the first business day and the last business day of the relevant purchase period. The fair values of stock purchase rights are estimated using the Black-Scholes-Merton option-pricing model. The fair value of the look-back provision plus the 15% discount is recognized on a graded-vesting basis as stock-based compensation expense over the purchase period.

For additional information related to stock-based compensation, please read *Note 13, Stock-Based Compensation* to the consolidated financial statements.

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 U.S. 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.

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.

During 2014, 2013 and 2012, the Company recognized rent expense and occupancy costs of $4.4 million, $3.4 million and $2.6 million, respectively.

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.

Subsequent Events

Subsequent events have been evaluated up through the date that these consolidated financial statements were filed and no material subsequent events were identified.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-15 which requires an entity s management to evaluate whether there are conditions or events,

considered in the aggregate, that raise substantial doubt about the entity s ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued. Substantial doubt about an entity s ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will not be able to meet its obligations as they become due within one year after the date that the financial statements are issued or available to be issued. If conditions or events raise substantial doubt about an entity s ability to continue as a going concern, but the substantial doubt is alleviated as a result of consideration of management s plans to mitigate those relevant conditions or events, the entity is required to disclose (1) principal conditions or events that raise substantial doubt about the entity s ability to continue as a going concern, (2) management s evaluation of the significance of those conditions or events in relation to the entity s ability to meet its obligations, and (3) management s plans that alleviate substantial doubt about the entity s ability to continue as a going concern. However, if conditions or events raise substantial doubt about an entity s ability to continue as a going concern, and substantial doubt is not alleviated after consideration of management s plans, an entity should include a statement in the footnote indicating that there is substantial doubt about the entity s ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued. ASU No. 2014-15 is effective for the annual period ending after December 15, 2016, with early adoption permitted. The Company has not yet adopted this standard.

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

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

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

Table of Contents

3. SIGNIFICANT AGREEMENTS

University of Western Australia

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 (the Amended and Restated UWA License Agreement). The Amended and Restated UWA License Agreement grants the Company specific rights to the treatment of DMD by inducing the skipping of certain exons. The Company s 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.

Under the Amended and Restated UWA License Agreement, the Company is required to meet certain performance diligence obligations related to development and commercialization of products developed under the license. The Company believes that it is currently in compliance with these obligations. In 2013, the Company made an initial upfront payment to UWA of $1.1 million upon execution of the Amended and Restated UWA License Agreement. The Company may be required to make additional payments to UWA of up to $6 million in aggregate based on successful achievement of certain regulatory and commercial milestones relating to eteplirsen and up to five additional product candidates and may also be required to pay royalties of 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. As of December 31, 2014, the Company is not under any current obligation to make royalty payments to UWA until achievement of the first commercial sale.

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 research grant of approximately $2.5 million 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 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, 2014, 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. To date, the Company has recognized less than $0.1 million as revenue and did not recognize any revenue for the years ended December 31, 2014, 2013 or 2012. The Company does not expect to receive any incremental funding under the grant and has deferred $3.3 million of previous receipts which are anticipated to be recognized as revenue upon resolution of outstanding performance obligations.

Isis Ercole Agreement

In May 2003, Ercole Biotechnology, Inc. (Ercole) and Isis Pharmaceuticals, Inc. (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 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.

Table of Contents

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, 2014, 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 and infectious disease 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 product developed under a licensed patent under this agreement. As of December 31, 2014, Isis has not made, and is not under any current obligation to make, any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. 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 expired 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.

4. FAIR VALUE MEASUREMENTS

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

	Fair Value Measurement as of December 31, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 47,740	$ 47,740	$	$
Commercial paper	2,997		2,997	
Government and government agency bonds	75,250		75,250	
Corporate bonds	58,546		58,546	
Certificates of deposit	647	647		
Total assets	$ 185,180	$ 48,387	$ 136,793	$

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

Table of Contents

	Fair Value Measurement as of December 31, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants	$	$	$	$
Total liabilities	$	$	$	$

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

The Company s assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the consolidated balance sheets as of December 31, 2014.

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

The Company s liabilities with fair value categorized as Level 3 within the fair value hierarchy consist of warrants issued in January and August 2009. The fair value of these liabilities is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs in the model. As of December 31, 2014, all outstanding warrants issued in January and August 2009 had been exercised or forfeited.

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

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

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

	As of December 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 73,551	$	$	$ 73,551
Commercial paper	2,997			2,997
Government and government agency bonds	75,289		(39)	75,250
Corporate bonds	58,602		(56)	58,546
Total	$ 210,439	$	$ (95)	$ 210,344
As reported:				
Cash and cash equivalents	73,551			73,551

Short-term investments	136,888		(95)	136,793
Total	$ 210,439	$	$ (95)	$ 210,344

Table of Contents

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

6. OTHER CURRENT ASSETS

The following table summarizes the Company s other current assets for each of the periods indicated:

	As of December 31, 2014	As of December 31, 2013
	(in thousands)	
Manufacturing-related deposits	$ 30,668	$
Prepaids	2,797	2,423
Other	1,571	638
Total other current assets	$ 35,036	$ 3,061

7. PROPERTY AND EQUIPMENT

Property and equipment are recorded at historical cost, net of accumulated depreciation. The following table summarizes components of property and equipment, net for each of the periods indicated:

	As of December 31,	
	2014	2013
	(in thousands)	
Land	$ 4,158	$
Building	12,617	1,856
Software and computer equipment	4,415	225
Lab equipment	11,772	7,728
Office equipment	2,713	1,207
Leasehold improvements	21,640	10,058
Construction in progress	1,082	11,303
Property and equipment, gross	58,397	32,377
Less accumulated depreciation	(19,896)	(17,328)
Property and equipment, net	$ 38,501	$ 15,049

For the years ended December 31, 2014, 2013 and 2012, depreciation expenses totaled $3.2 million, $0.8 million and $1.0 million, respectively.

Andover, Massachusetts Facility

On May 22, 2014, the Company entered into a Purchase and Sales Agreement with Eisai, Inc. to acquire certain real and personal property located in Andover, Massachusetts. The aggregate purchase price, including

Table of Contents

certain fees and taxes was approximately $15.1 million, of which approximately $10.1 million was paid at closing and the remaining $5.0 million will be paid in two installments by July 15, 2015 and January 15, 2016. On July 15, 2014, the closing of the purchase of the real and personal property was completed. In connection with this transaction, as of December 31, 2014, the Company recorded $4.2 million as land and $10.8 million as building on the consolidated balance sheets.

8. ACCRUED EXPENSES

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

	As of December 31, 2014	As of December 31, 2013
	(in thousands)	
Accrued contract manufacturing costs	$ 3,271	$ 1,414
Accrued facility-related costs	300	2,843
Accrued contract research costs	3,782	2,785
Accrued employee compensation costs	6,170	5,048
Accrued professional fees	3,403	1,235
Other	440	1,276
Total accrued expenses	$ 17,366	$ 14,601

9. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in January and August 2009 were classified as liabilities as opposed to equity due to their settlement terms which required settlement in registered shares. The outstanding warrants classified as liabilities were recorded on the consolidated balance sheets and adjusted to fair value at each financial reporting period, with changes in the fair value being recorded as Loss on change in warrant valuation in the consolidated statements of operations and comprehensive loss. Fair value was determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs used in the model. As of December 31, 2014, there were no outstanding warrants as all warrants issued in January and August 2009 have been exercised or have expired.

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

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Balance at beginning of the period	$ 9,006	$ 65,193	$ 5,446
Increase in value of warrants	2,779	22,027	91,938
Reclassification to stockholders equity upon exercise of warrants	(11,785)	(78,214)	(32,191)
Balance at end of the period	$	$ 9,006	$ 65,193

Table of Contents

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

	For the Year Ended December 31,					
	2014		2013		2012	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Warrants outstanding at beginning of the period	791,508	$ 10.05	3,127,618	$ 8.48	4,867,477	$ 9.54
Exercised	(765,915)	10.12	(2,336,110)	7.96	(1,739,859)	11.81
Canceled or expired	(25,593)	7.92				
		$	791,508	$ 10.05	3,127,618	$ 8.48

10. INDEBTEDNESS

Notes payable

In connection with the acquisition of the Andover, Massachusetts facility, the Company issued a promissory note with a principal amount of $5.0 million. The promissory note with interest on the outstanding balance at the lowest short-term applicable federal rate per annum will be paid in two installments on July 15, 2015 and January 15, 2016. As a result, the Company recorded $2.5 million as current portion of notes payable and $2.3 million as notes payable on the consolidated balance sheets as of December 31, 2014.

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, 2014, these loans had unpaid principal balances of $1.0 million and $0.6 million, for a total indebtedness of $1.6 million. For each the three years ended December 31, 2014, 2013 and 2012, the Company incurred interest expense on these loans of approximately $0.1 million.

The following table summarizes the total payments under the Company s debt arrangements:

	Long-term Debt (1)	Notes Payable (1)	Total
		(in thousands)	
2015	$ 171	$ 2,515	$ 2,686
2016	171	2,504	2,675
2017	171		171
2018	171		171
2019	171		171
Thereafter	1,224		1,224
Total Payments	$ 2,079	$ 5,019	$ 7,098

(1) Interest is included

11. EQUITY FINANCING

In September and October 2012, the Company sold 2.0 million shares of its common stock through an At-the-Market (ATM) offering (2012 ATM) 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.

Table of Contents

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

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

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

12. 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 on a gross basis in the consolidated statement of operations and comprehensive loss. In the periods presented, substantially all of the revenue the Company generated was derived from research contracts and grants from the U.S. government. As of December 31, 2014, the Company had completed all development activities of its contracts with the Department of Defense (DoD).

The following table sets forth the revenue from each of the Company s contracts with the U.S. government and other revenue for each of the periods indicated:

	For the Year Ended December 31		
	2014	2013	2012
		(in thousands)	
July 2010 Agreement (*Ebola and Marburg Intravenous Administration)*	$ 6,816	$ 9,064	$ 36,557
June 2010 Agreement (*H1N1/Influenza*)		427	
August 2012 Agreement (*Intramuscular administration*)		2,791	673
European Union SKIP-NMD Agreement (*DMD*)	1,432	1,263	
Children s National Medical Center Agreement (DMD)	659	674	
Carolinas Medical Center Agreement (DMD)	850		
Other Agreements			99
Total	$ 9,757	$ 14,219	$ 37,329

July 2010 Agreement (Ebola and Marburg Intravenous Administration)

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

The Marburg portion of the contract was structured into four segments and had an aggregate remaining period of performance spanning approximately four years if the DoD exercised its options for all segments. Activities under the first segment began in July 2010 and included preclinical studies and Phase I studies in

Table of Contents

healthy volunteers. In February 2014, the Company announced positive safety results from the Phase I multiple ascending dose study of AVI-7288. In July 2014, the Marburg portion of the agreement expired. For the years ended December 31, 2014, 2013 and 2012, the Company recognized $6.8 million, $9.1 million and $36.6 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of December 31, 2014 and only revenue for contract finalization, if any, is expected in the future.

June 2010 Agreement (H1N1/Influenza)

In June 2010, the Company entered into an agreement 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 (TMT) of the DoD. The period of performance for this agreement ended in June 2011. The Company recognized $0.4 million associated with this agreement for the year ended December 31, 2013, which was the result of an indirect rate adjustment.

August 2012 Agreement (Intramuscular administration)

In August 2012, the Company was awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, the Company s candidate for treatment of the Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. Accordingly, no revenue was recognized since the conclusion of the contract. For the years ended December 31, 2013 and 2012, the Company recognized $2.8 million and $0.7 million, respectively, as revenue under this agreement.

European Union SKIP-NMD Agreement (DMD)

In November 2012, the Company entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic. For the years ended December 31, 2014 and 2013, the Company recognized $1.4 million and $1.3 million, respectively, as revenue under this agreement. The majority of the revenue under this contract has been recognized as of December 31, 2014 and only revenue for contract finalization, if any, is expected in the future.

Children s National Medical Center Agreement (DMD)

In July 2013, the Company entered into an agreement totaling $1.3 million to provide drug product to Children s National Medical Center to conduct research related to the Company s DMD program. During each of the years ended December 31, 2014 and 2013, the Company recognized $0.7 million as revenue under the agreement.

Carolinas Medical Center Agreement (DMD)

The Company entered into a collaboration agreement with Carolinas Medical Center (CMC) to co-develop one of the Company s DMD programs. Under the agreement, CMC was obligated to reimburse certain preclinical costs incurred by the Company. All preclinical work was completed and the Company recognized revenue of $0.9 million for the twelve months ended December 31, 2014.

13. STOCK-BASED COMPENSATION

In June 2011, the Company s stockholders approved the 2011 Equity Incentive Plan (2011 Plan). The 2011 Plan, which authorized 13 million shares of common stock to be issued, 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, 2014, 1.8 million shares of common stock remain available for future grant.

Table of Contents

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, 2014, 203,710 shares of common stock remain available for future grant.

In September 2014, the Company s stockholders approved the 2014 Employment Commencement Incentive Plan (the 2014 Plan) with 640,000 shares of common stock available to be issued. As of December 31, 2014, 640,000 shares of common stock remain available for future grant.

Stock Options

In general, stock options 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.

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

	For the Year Ended December 31,		
	2014	2013	2012
Risk-free interest rate (1)	1.4 1.7%	0.7 1.7%	0.6 1.1%
Expected dividend yield (2)			
Expected lives (3)	4.7 4.9 years	4.8 5.0 years	4.8 5.3 years
Expected volatility (4)	93.0 103.0%	80.0 90.7%	79.7 108.6%

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

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

Additionally, the Company is required to estimate potential forfeiture of stock grants and adjust stock-based 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.

Table of Contents

The following tables summarize the Company s stock option activity for each of the periods indicated:

	For the Year Ended December 31,					
	2014		2013		2012	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	4,190,367	$ 23.46	2,522,522	$ 11.76	2,417,659	$ 11.18
Granted	1,694,560	25.67	2,283,719	34.18	1,269,470	12.92
Exercised	(86,007)	11.40	(241,056)	11.31	(371,353)	10.18
Canceled or expired	(582,717)	22.79	(371,818)	16.83	(793,254)	12.59
Grants outstanding at end of the period	5,216,203	$ 24.45	4,190,367	$ 23.46	2,522,522	$ 11.76
Grants exercisable at end of the period	2,019,514	$ 18.69	1,051,329	$ 11.91	615,394	$ 12.71
Grants vested and expected to vest at end of the period	4,462,100	$ 23.27	3,467,069	$ 21.50	2,343,086	$ 11.73

The weighted-average fair value per share of stock options granted during the years ended December 31, 2014, 2013 and 2012 was $18.59, $22.86 and $9.54, respectively.

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
Options outstanding at December 31, 2014	$ 7,794	8.04
Options exercisable at December 31, 2014	$ 5,973	7.04
Options vested and expected to vest at December 31, 2014	$ 7,671	7.94

The following table summarizes the Company s stock options vested and exercised for each of the periods indicated:

	For the Year Ended December 31,		
	2014	2013	2012
	(in thousands)		
Aggregate grant date fair value of stock options vested	$ 17,672	$ 4,872	$ 3,749
Aggregate intrinsic value of stock options exercised	$ 1,497	$ 5,444	$ 4,964

Stock Options with Service- and Performance-based Conditions

In June 2013, the Company granted to executives approximately 0.4 million stock options with service- and performance-based conditions. Vesting is achieved based upon various regulatory filings including new drug application (NDA) for eteplirsen and investigational new drug (IND) submissions for other drug candidates and continuing service over a four-year period. Through the submission of two IND applications during 2014, 30% of performance awards were triggered to be eligible to vest subject to the remaining service conditions of the awards. For the year ended December 31, 2014, the Company has recognized approximately $1.2 million in stock-based compensation expense related to the options with performance-based criteria.

As of December 31, 2014, the total stock-based compensation expense related to non-vested awards with only service-vesting conditions not yet recognized is approximately $40.9 million and those with service- and performance-based conditions approximates $9.1 million.

Table of Contents

Restricted Stock Awards

The Company grants RSAs to members of its board of directors. The following table summarizes the Company s RSA activity for each of the periods indicated:

	For the Year Ended December 31,					
	2014		2013		2012	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	6,000	$ 34.92	4,998	$ 10.08	5,000	$ 8.46
Granted			6,000	34.92	4,998	10.08
Vested			(4,998)	10.08	(5,000)	8.46
Canceled or expired						
Grants outstanding at end of the period	6,000	$ 34.92	6,000	$ 34.92	4,998	$ 10.08

Restricted Stock Units

The Company granted RSUs to employees in 2012. The following table summarizes the Company s RSU activity for each of the periods indicated:

	For the Year Ended December 31,					
	2014		2013		2012	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	6,507	$ 5.40	38,260	$ 6.32		$
Granted					39,877	6.28
Vested	(6,507)	5.40	(31,379)	6.52		
Canceled or expired			(374)	5.40	(1,617)	5.40
Grants outstanding at end of the period		$	6,507	$ 5.40	38,260	$ 6.32

Stock Appreciation Rights

The Company issues 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.

Table of Contents

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

	For the Year Ended December 31,					
	2014		2013		2012	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	170,000	$ 18.18	170,000	$ 18.18		$
Granted					170,000	18.18
Grants outstanding at end of the period	170,000	$ 18.18	170,000	$ 18.18	170,000	$ 18.18
Grants exercisable at end of the period	92,916	$ 17.80	50,416	$ 17.48		$
Grants vested and expected to vest at end of the period	170,000	$ 18.18	170,000	$ 18.18	170,000	$ 18.18

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
SARs outstanding at December 31, 2014	$ 307	7.76
SARs exercisable at December 31, 2014	$ 179	7.76
SARs vested and expected to vest at December 31, 2014	$ 307	7.76

Employee Stock Purchase Plan (ESPP)

Under the Company s ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company s common stock on the first business day and the last business day of the relevant purchase period. The 24-month award period will end on August 31, 2016. For the year ended December 31, 2014, 46,290 shares of the Company s common stock were purchased for total proceeds of approximately $1.0 million.

Stock-based Compensation Expense

For the years ended December 31, 2014, 2013 and 2012, total stock-based compensation expense was $20.3 million, $11.1 million and $3.1 million, respectively. The following table summarizes stock-based compensation expense by function included within the consolidated statements of operations and comprehensive loss:

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Research and development	$ 8,269	$ 3,888	$ 1,173
General and administrative	12,076	7,239	1,905
Total	$ 20,345	$ 11,127	$ 3,078

Table of Contents

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

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Stock options	$ 18,388	$ 9,632	$ 2,867
Restricted stock awards	204	149	38
Restricted stock units	1	269	63
Stock appreciation rights	587	593	110
Employee stock purchase plan	1,165	484	
Total	$ 20,345	$ 11,127	$ 3,078

14. 401 (K) PLAN

The Company sponsors a 401 (k) Plan (the Plan) which is a defined contribution plan. It is available to all employees who are age 21 or older. Participants may make voluntary contributions and the Company makes matching contributions according to the Plan s matching formula. All matching contributions fully vest after one year of service. The expense related to the Plan primarily consists of the Company s matching contributions.

Expense related to the Plan totaled $0.6 million, $0.4 million and $0.3 million for the years ended December 31, 2014, 2013 and 2012, respectively.

15. RESTRUCTURING

In December 2011, the Company restructured its operations by reducing its workforce by 35 employees. 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 an employee through the second quarter of 2014. As of June 30, 2014, the program was completed.

The following table summarizes changes in the liability and the balance at year end related to these restructuring plans for each of the periods indicated.

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Balance at beginning of the period	$ 44	$ 185	$ 828
Restructuring charge	14	764	185
Payments	(58)	(905)	(828)
Balance at ending of the period	**$**	**$ 44**	**$ 185**

Table of Contents

The following table summarizes restructuring expense by function for each of the periods indicated.

	For the Year Ended December 31,		
	2014	2013	2012
		(in thousands)	
Research and development	$ 14	$ 414	$ 69
General and administrative		350	116
Total	**$ 14**	**$ 764**	**$ 185**

16. INCOME TAXES

As of December 31, 2014, the Company had federal and state net operating loss carryforwards of $324.0 million and $220.9 million, respectively, available to reduce future taxable income, which expire between 2015 and 2034. 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. Additionally, the Company has $25.3 million and $5.0 million of federal and state research and development credits, respectively, available to offset future taxable income. These federal and state research and development credits begin to expire between 2018 and 2034 and between 2015 and 2029, respectively. Approximately $13.0 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 accumulated deficit 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, and differences in the recognition of stock-based compensation.

The Company had gross deferred tax assets of $165.8 million and $133.6 million at December 31, 2014 and 2013, respectively, primarily from U.S. federal and state net operating loss carryforwards, U.S. federal and state research and development credit carryforwards, stock-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:

	As of December 31,	
	2014	2013
	(in thousands)	
Net operating loss carryforwards	$ 115,699	$ 94,170
Difference in depreciation and amortization	2,800	2,492
Research and development tax credits	29,127	23,599
Stock-based compensation	13,637	9,036
Deferred rent	2,864	2,849
Deferred revenue	1,213	1,219
Other	413	208
Gross deferred tax assets	165,753	133,573
Valuation allowance	(165,753)	(133,573)
Net deferred tax asset	$	$

The net change in the valuation allowance for deferred tax assets was an increase of $32.2 million and $19.4 million for the years ended December 31, 2014 and 2013, respectively, mainly due to the increase in the net operating loss carryforwards, stock-based compensation and research and development tax credits.

Table of Contents

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

	For the Year Ended December 31,		
	2014	2013	2012
Federal income tax rate	34.0%	34.0%	34.0%
Research and development tax credits	2.4	1.4	(0.6)
Valuation allowance	(21.6)	(12.4)	(7.5)
Permanent Differences	(2.4)	(8.8)	(25.9)
Foreign rate differential	(12.4)	(14.2)	
Effective tax rate	%	%	%

Permanent differences affecting the Company s effective tax rate include 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. For the years ended December 31, 2014 and 2013, Sarepta International C.V. incurred $48.5 million and $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, 2014 or December 31, 2013, and has not recognized interest and/or penalties in the statement of operations for years ended December 31, 2014, 2013 or 2012. The Company has not recognized any liability for unrecognized tax benefits.

17. NET LOSS PER SHARE

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

	For the Year Ended December 31,		
	2014	2013	2012
	(in thousands, except for per share amounts)		
Net loss	$ (135,789)	$ (111,985)	$ (121,287)
Weighted-average number of shares of common stock and common stock equivalents outstanding:			
Weighted-average number of common stock outstanding for computing basic net loss per share	40,026	33,850	23,602
Dilutive effect of outstanding warrants and stock awards after application of the treasury stock method*			
Weighted-average number of common stock outstanding for computing diluted net loss per share	40,026	33,850	23,602
Net loss per share basic and diluted	$ (3.39)	$ (3.31)	$ (5.14)

* Warrants, stock options, restricted stock units and stock appreciation rights to purchase approximately 5,386,203, 5,158,382 and 5,858,000 shares of common stock as of December 31, 2014, 2013 and 2012, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

Table of Contents

18. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement (Cambridge lease) for its headquarters located in Cambridge, Massachusetts. As of December 31, 2014, the Company had entered into five amendments to the Cambridge lease, increasing its total rental space for its headquarters to 77,390 square feet. The Cambridge lease and its amendments will expire in January 2021. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit (CD) to meet the requirement and it was recorded as a long-term restricted investment in the consolidated balance sheets as of December 31, 2014.

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

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

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

The following table summarizes the aggregate non-cancelable future minimum payments under the Company s leases:

	As of December 31, 2014 (in thousands)
2015	$ 4,382
2016	4,612
2017	4,729
2018	4,846
2019	4,966
Thereafter	5,628
Total minimum lease payments	$ 29,163

Royalty Obligations

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

Milestone Obligations

The Company has license agreements for which it is obligated to pay development and commercial milestones as a product candidate proceeds from the submission of an IND application through approval for commercial sale. There were no significant milestone payments under these agreements for the years ended December 31, 2014, 2013 or 2012.

Table of Contents

Under the collaboration and license agreement with Isis-Ercole, the Company may be obligated to make up to $26.8 million in milestone payments. As of December 31, 2014, the Company had not made any payments under this agreement and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, Isis may be obligated to make milestone payments to the Company of up to $21.1 million in the aggregate for each product developed under a licensed patent under this agreement. As of December 31, 2014, Isis has not made and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied.

In April 2013, the Company and UWA entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated. Under the terms of this agreement, UWA granted the Company an exclusive license to certain UWA intellectual property rights in exchange for up to $7.1 million in up-front, development and commercial milestone payments. For the years ended December 31, 2014 and 2013, the Company recorded $0 and $1.0 million, respectively, relating to certain up-front payments required under the agreement as research and development expense in the consolidated statements of operations and comprehensive loss.

In March 2014, the Company entered into a patent assignment agreement with a group of scientists (collectively, Assignors). Under the terms of the agreement, the Assignors transferred to the Company all rights, title and interest in certain patent rights as well as technical information related to the patents. The Company may be obligated to make up to $2.7 million in development and commercial milestone payments. For the year ended December 31, 2014, the Company recorded $0.3 million relating to an up-front payment as research and development expense in the consolidated statement of operations and comprehensive loss.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs consolidated amended complaint, filed on July 21, 2014, seeks to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleges that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seeks damages in an unspecified amount. Pursuant to the court s June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, which remains pending. In addition, another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Chris Garabedian, and Sandy Mahatme, 1:14-cv-14318, asserting violations of Section 10(b) of the Exchange Act and SEC Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme. The plaintiff in this case alleges that the defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company s data for submission of an new drug application for eteplirsen and the likelihood of the FDA accepting a new drug application based on that data. Plaintiff seeks compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta will move to dismiss the complaint. Additionally, on September 23, 2014, a derivative suit was filed against the Company s Board of Directors with the Court of Chancery of the State of Delaware (Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157). The claims allege, among other things, that (i) the Company s non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the

Table of Contents

compensation for the Company s CEO was also excessive and such fees were the basis for the CEO not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, are disgorgement and rescindment of excessive or unfair payments and equity grants to the CEO and directors, unspecified damages plus interest, a class action declaration for the suit, declaring approval of the Company s Amended and Restated 2011 Equity Plan at the 2013 meeting ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff s attorney fees. Given the relatively early stages of the proceedings in the *Corban* and *Kader* suits, 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. We do not believe that disposition of the *McDonald* suit should have a material financial impact on the Company.

Purchase Commitments

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

	As of December 31, 2014 (in thousands)
2015	$ 56,232
2016	40,188
2017	23,940
2018	14,260
2019	5,705
Total purchase commitments	$ 140,325

In February 2013, the Company issued two letters of credit totaling $7.3 million to a contract manufacturer in connection with certain manufacturing agreements. The obligations secured by the letters of credit are fulfilled upon payment for certain minimum volume commitments and construction milestones. To meet the requirement of the letters of credit, the Company purchased $7.3 million in CDs. As of December 31, 2014, both CDs were released and matured.

19. FINANCIAL INFORMATION BY QUARTER (UNAUDITED)

	2014 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenue from research contracts and other grants	$ 27	$ 1,059	$ 2,583	$ 6,088
Operating expenses:				
Research and development	30,832	21,852	20,641	20,906
General and administrative	13,917	12,882	12,213	10,303
Total operating expenses	44,749	34,734	32,854	31,209
Operating loss	(44,722)	(33,675)	(30,271)	(25,121)
Other income (loss):				
Interest income and other, net	306	193	181	99
Gain (loss) on change in warrant valuation		4,256	(3,784)	(3,251)

Total other income (loss)	306	4,449	(3,603)	(3,152)
Net loss	$ (44,416)	$ (29,226)	$ (33,874)	$ (28,273)
Net loss per share basic and diluted	$ (1.08)	$ (0.71)	$ (0.85)	$ (0.75)
Shares used in per share calculations basic and diluted	41,304	41,066	39,862	37,821

Table of Contents

	2013 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenue from research contracts and other grants	$ 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
Total operating expenses	35,475	29,101	20,038	19,889
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)
Total other income (loss)	24,029	(17,097)	(1,964)	(26,669)
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