

AERIE PHARMACEUTICALS INC

Form 10-K

February 27, 2015

Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

(Mark One)

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

Commission File Number: 001-36152

Aerie Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware

(State or other jurisdiction of incorporation or organization)

2030 Main Street, Suite 1500

Irvine, California 92614

(949) 526-8700

(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

20-3109565

(IRS Employer

Identification No.)

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

Title of Each Class

Common Stock, \$0.001 par value per share

Name of Each Exchange on Which Registered

NASDAQ Global 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

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

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

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

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

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

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

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

The aggregate market value of the voting stock held by non-affiliates of the registrant on June 30, 2014, based upon the closing price of \$24.77 of the registrant's common stock as reported on the NASDAQ Global Market, was \$172,438,000. Shares of the registrant's common stock held by each officer and director and each person known to the registrant to own 10% or more of the outstanding voting power of the registrant have been excluded because such persons may be deemed affiliates. This determination of affiliate status is not a determination for other purposes. As of February 20, 2015, the registrant had 24,046,939 shares of common stock, \$0.001 par value, issued and outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement (the "Proxy Statement") for the 2015 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K. The Proxy Statement will be filed with the Securities and Exchange Commission (the "SEC") within 120 days of the registrant's fiscal year ended December 31, 2014.

Table of Contents

TABLE OF CONTENTS

	Page
<u>SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS</u>	<u>ii</u>
PART I	
Item 1. <u>Business</u>	<u>1</u>
Item 1A. <u>Risk Factors</u>	<u>30</u>
Item 1B. <u>Unresolved Staff Comments</u>	<u>58</u>
Item 2. <u>Properties</u>	<u>58</u>
Item 3. <u>Legal Proceedings</u>	<u>58</u>
Item 4. <u>Mine Safety Disclosures</u>	<u>58</u>
PART II	
Item 5. <u>Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities</u>	<u>59</u>
Item 6. <u>Selected Financial Data</u>	<u>62</u>
Item 7. <u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>63</u>
Item 7A. <u>Quantitative and Qualitative Disclosures About Market Risk</u>	<u>75</u>
Item 8. <u>Financial Statements and Supplementary Data</u>	<u>75</u>
Item 9. <u>Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</u>	<u>76</u>
Item 9A. <u>Controls and Procedures</u>	<u>76</u>
Item 9B. <u>Other Information</u>	<u>77</u>
PART III	
Item 10. <u>Directors, Executive Officers and Corporate Governance</u>	<u>78</u>
Item 11. <u>Executive Compensation</u>	<u>78</u>
Item 12. <u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	<u>78</u>
Item 13. <u>Certain Relationships and Related Transactions, and Director Independence</u>	<u>78</u>
Item 14. <u>Principal Accountant Fees and Services</u>	<u>78</u>
PART IV	
Item 15. <u>Exhibits and Financial Statement Schedules</u>	<u>79</u>

Table of Contents

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This report contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the “Securities Act”), and Section 21E of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). We may, in some cases, use terms such as “predicts,” “believes,” “potential,” “proposed,” “continue,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “would,” “could,” “might,” “will,” “should,” “exploring,” “pursuing” or other similar terms to convey uncertainty of future events or outcomes to identify these forward-looking statements.

Forward-looking statements appear in a number of places throughout this report and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things:

- the success, timing and cost of our ongoing and anticipated preclinical studies and clinical trials for our current and potential future product candidates, including statements regarding the timing of initiation and completion of the studies and trials;
- our expectations regarding the clinical effectiveness of our product candidates and results of our clinical trials;
- the timing of and our ability to obtain and maintain U.S. Food and Drug Administration (“FDA”) or other regulatory authority approval of, or other action with respect, to our product candidates;
- our expectations related to the use of proceeds from our initial public offering (“IPO”) in October 2013 and the issuance and sale of the 2014 Convertible Notes (as defined herein) in September 2014;
- our estimates regarding anticipated capital requirements and our needs for additional financing;
- the commercial launch and potential future sales of our current or any other future product candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- third-party payor reimbursement for our product candidates;
- the glaucoma patient market size and the rate and degree of market adoption of our product candidates by eye-care professionals and patients;
- the timing, cost or other aspects of the commercial launch of our product candidates;
- our plans to pursue development of our product candidates for additional indications and other therapeutic opportunities;
- the potential advantages of our product candidates;
- our plans to explore possible uses of our existing proprietary compounds beyond glaucoma;
- our ability to protect our proprietary technology and enforce our intellectual property rights;
- our expectations regarding collaborations, licensing, acquisitions and strategic operations, including our ability to in-license or acquire additional ophthalmic products or product candidates; and
- our mission to build a major ophthalmic pharmaceutical company.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics and industry change, and depend on regulatory approvals and economic and other environmental circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated.

We discuss many of these risks in greater detail under the heading “Risk Factors” in Part I, Item 1A of this report and elsewhere in this report. You should not rely upon forward-looking statements as predictions of future events.

Although we believe that we have a reasonable basis for each forward-looking statement contained in this report, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this report. In addition, even if our results of operations, financial condition and liquidity, and events in the industry in which we operate are consistent with the forward-looking statements contained in this report, they may not be predictive of results or developments in future periods.

Any forward-looking statements that we make in this report speak only as of the date of this report. Except as required by law, we are under no duty to update or revise any of the forward-looking statements, whether as a result of new information, future events or otherwise, after the date of this report.

Table of Contents

PART I

ITEM 1. BUSINESS

Overview

We are a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with glaucoma and other diseases of the eye. Our strategy is to advance our product candidates, including triple-action Rhopressa™ and quadruple-action Roclatan™, to regulatory approval, and commercialize these products ourselves in North American markets and possibly Europe. We plan to build a commercial team of approximately 100 sales representatives to target approximately 10,000 high prescribing eye-care professionals throughout North America. For certain key markets outside North America, including Japan, emerging markets and possibly Europe, we intend to explore partnership opportunities through collaboration and licensing arrangements. We plan to further maximize our commercial potential by identifying and advancing additional product candidates, both through our internal discovery efforts and through possible in-licensing or acquisitions of additional ophthalmic products or product candidates that would complement our current product portfolio. We completed our IPO in October 2013 and raised net proceeds of approximately \$68.3 million. In September 2014, we raised additional net proceeds of approximately \$124.1 million through the sale and issuance of privately placed senior secured convertible notes.

Our senior leadership team has extensive experience in the ophthalmology market and has overseen the development and commercialization at major pharmaceutical companies of several successful ophthalmic products. If our products are approved and we are commercially successful, we believe Aerie could become a market-leading ophthalmic pharmaceutical company.

Our lead product candidate, once-daily, triple-action Rhopressa™, successfully completed a Phase 2b clinical trial in patients with open-angle glaucoma and ocular hypertension in May 2013. Phase 3 registration trials commenced in July 2014. Our Phase 3 registration trial (“Rocket 1”) and a second Phase 3 registration trial (“Rocket 2”) will measure efficacy over three months. The primary efficacy endpoint of the trials is to demonstrate non-inferiority of Rhopressa™ compared to timolol for the lowering of IOP. Timolol is the most widely used comparator in registration trials for lowering of intraocular pressure, or IOP. Rocket 2 is also designed to assess safety over 12 months. In addition, we are conducting a one year, safety-only study in Canada, named “Rocket 3.” Pending successful advancement of the Phase 3 registration trials, three-month efficacy results are expected in the middle of the second quarter 2015 for Rocket 1 and in mid-2015 for Rocket 2.

We are developing Rhopressa™ as the first of a new class of compounds that is designed to lower IOP in patients through novel mechanisms of action, or MOAs. We believe that, if approved, Rhopressa™ will represent the first new MOAs for lowering IOP in patients with glaucoma in over 20 years. Based on clinical data to date, we expect Rhopressa™ to compete within the prostaglandin analogue, or PGA, market segment due to its equivalent or potentially better efficacy for patients with IOP of 26 millimeters of mercury, or mmHg, or below at the time of diagnosis, which we refer to as “low to moderately elevated” IOP, while also targeting the diseased tissue responsible for elevated IOP. Approximately 80% of glaucoma patients have low to moderately elevated IOP at the time of diagnosis. Furthermore, if approved, we expect Rhopressa™ to compete against non-PGA products as a preferred add-on therapy to PGAs, due to its strong and consistent IOP-lowering effect with once-daily dosing relative to currently marketed non-PGA products. In addition, we expect Rhopressa™ to become a preferred therapy where PGAs are contraindicated, for patients who do not respond to PGAs, for patients who have IOPs below 21 mmHg but nevertheless present with glaucomatous damage to the optic nerve, which is commonly referred to as “low-tension” glaucoma, as well as for patients who choose to avoid the cosmetic issues associated with PGAs.

Our second product candidate, once-daily, quadruple-action Roclatan™, is a single drop fixed-dose combination of Rhopressa™ and latanoprost, the most commonly prescribed drug for the treatment of patients with glaucoma. Roclatan™ successfully completed a Phase 2b clinical trial in patients with open-angle glaucoma and ocular hypertension in June 2014 and achieved its primary efficacy endpoint on day 29 and statistical superiority over individual components at all timepoints. We believe Roclatan™ has the potential to provide a greater IOP-lowering effect than any currently

approved glaucoma product. Therefore, we believe Roclatan™ could compete with both PGA and non-PGA therapies and become the product of choice for patients requiring maximal IOP lowering. We expect Phase 3 registration trials to commence in mid-2015. Preparatory steps for such trials are well underway.

Our mission is to build a major ophthalmic pharmaceutical company. In addition to our primary product candidates, Rhopressa™ and Roclatan™, we are also exploring the longer-term impact of Rhopressa™ and Roclatan™ on the diseased trabecular meshwork, as well as potential neuroprotective benefits, and evaluating possible uses of our existing proprietary portfolio of Rho Kinase inhibitors beyond glaucoma. We recently issued a research update on preclinical results demonstrating the potential for Rhopressa™ to have disease-modifying activity in glaucoma by stopping fibrosis in the trabecular meshwork,

Table of Contents

and also increasing perfusion in the trabecular outflow pathway thus increasing the delivery of nutrients to the diseased tissue. Additionally, an early-stage molecule, AR-13154, has shown the preclinical potential to decrease lesions in wet age-related macular degeneration at numerically higher levels than current market leading products. Our strategy includes developing our business outside of North America, including potentially obtaining clinical approval on our own for our lead compounds in Europe and possibly Japan. Regarding commercialization strategy, if our products are successful, we may potentially commercialize ourselves or with a partner in Europe, and potentially with a partner in Japan. As we prepare for foreign-based activities, we are evaluating optimized supply chain configurations and domicile alternatives for our non-U.S. intellectual property.

We may license, acquire or develop additional product candidates to broaden our presence in ophthalmology. We are continuing to explore collaboration opportunities for new ophthalmic products, delivery alternatives and new therapeutic areas, including gene therapy. However, we have no present plans, agreements or commitments with respect to any potential acquisition, investment or license related to any such additional product candidates.

Glaucoma is one of the largest segments in the global ophthalmic market. In 2013, branded and generic glaucoma product sales exceeded \$4.5 billion in the United States, Europe and Japan in aggregate, according to IMS.

Prescription volume for glaucoma products in the United States alone exceeded 31 million in 2013 and is expected to grow, driven in large part by the aging population. The PGA and non-PGA market segments each represent approximately half of the prescription volume in the glaucoma market, as shown in the following pie chart, which is based on IMS data.

According to the National Eye Institute, it is estimated that over 2.7 million people in the United States suffer from glaucoma, a number that is expected to reach 4.3 million by 2030. Furthermore, The Eye Diseases Prevalence Research Group has estimated that only half of the nation's glaucoma sufferers know that they have the disease. Glaucoma is a progressive and highly individualized disease, in which elevated levels of IOP are associated with damage to the optic nerve, resulting in irreversible vision loss and potentially blindness. Patients may suffer the adverse effects of glaucoma across a wide range of IOP levels, including within the "normotensive" range of 10 to 21 millimeters of mercury, or mmHg, which is generally accepted as the level of IOP in healthy individuals. There are multiple factors that can contribute to an individual getting glaucoma, including age, family history and ethnicity. For example, there generally is a higher incidence and severity of the disease in African-American and Hispanic populations. Based on data from the Baltimore Eye Survey, approximately 80% of glaucoma patients have low to moderately elevated IOP at the time of diagnosis and approximately 60% of glaucoma patients have IOP of 21 mmHg or below at the time of diagnosis. Additionally, in Japan, the Tajimi Study found that approximately 90% of glaucoma patients had IOP of 21 mmHg or below at the time of diagnosis. In clinical trials to date, Rhopressa™ has demonstrated the ability to provide consistent IOP lowering across all tested baseline IOP levels, which we believe differentiates it from currently marketed drugs that have shown reduced efficacy at lower baseline IOPs.

Glaucoma is treated by the reduction of IOP, which has been shown to slow the progression of vision loss. In a healthy eye, fluid is continuously produced and drained in order to maintain pressure equilibrium and provide nutrients to the eye tissue. The FDA recognizes sustained lowering of IOP as the primary clinical endpoint for the approval of drugs to treat patients with

Table of Contents

glaucoma and ocular hypertension. The primary drainage mechanism of the eye is the trabecular meshwork, or TM, which accounts for approximately 80% of fluid drainage, while the secondary drainage mechanism, the uveoscleral pathway, is responsible for the remaining drainage. In glaucoma patients, damage to the TM results in insufficient drainage of fluid from the eye, which causes increased IOP and damage to the optic nerve. In addition to eye fluid production and drainage through the TM and uveoscleral pathway, episcleral venous pressure, or EVP, makes a significant contribution to IOP. EVP represents the pressure of the blood in the episcleral veins of the eye where the eye fluid drains into the bloodstream. Historical studies have shown that EVP accounts for approximately half of IOP in normotensive subjects and approximately one-third of IOP in patients with pressures of 24 to 30 mmHg. When EVP is lowered, fluid is able to flow more freely from the eye. Drugs that lower IOP without lowering EVP are most effective at high IOPs, where EVP is believed to contribute less to IOP, and are less effective at lower IOPs, where EVP is seen to account for a larger portion of IOP.

Once glaucoma develops, it is a chronic condition that requires life-long treatment. The initial treatment for glaucoma patients is typically the use of prescription eye drops. PGAs have become the most widely prescribed glaucoma drug class. The most frequently prescribed PGA is once-daily latanoprost. The most commonly prescribed non-PGA drugs belong to the beta blocker class. The most frequently prescribed beta blocker is twice-daily timolol. Other non-PGA drug classes include the alpha agonists and carbonic anhydrase inhibitors. When PGA monotherapy is insufficient to control IOP or contraindicated due to concerns about side effects, non-PGA products are used either as add-on therapy to the PGA or as an alternative monotherapy. It is estimated that up to 50% of glaucoma patients receiving PGA monotherapy require add-on therapy within two years of initial prescription of the drug, in order to maintain adequate control of IOP.

Our product candidates represent a new class of drugs utilizing novel MOAs that are applied topically as once-daily eye drops. Currently approved drugs mainly reduce IOP by increasing fluid outflow through the eye's secondary drain with once-daily dosing or reducing fluid inflow by decreasing fluid production with multiple doses per day. Rhopressa™ lowers IOP through a triple MOA that (i) relaxes the contracted tissue of the TM to improve fluid outflow through the eye's primary drain, (ii) decreases fluid production in the eye and (iii) lowers EVP, an MOA that we believe further differentiates Rhopressa™ from currently marketed glaucoma products. Roclatan™, our quadruple-action fixed-combination product candidate, combines the triple MOA of Rhopressa™ with latanoprost, a PGA that increases fluid drainage through the uveoscleral pathway.

We believe there are significant unmet needs in the glaucoma market and that eye-care professionals are eager for new therapy choices. None of the commonly prescribed PGAs or non-PGAs target the TM, the diseased tissue responsible for elevated IOP in glaucoma and the eye's primary drain. Moreover, PGAs have side effects, contraindications and reduced efficacy in patients with low to moderately elevated IOPs relative to patients with higher IOPs. Non-PGAs are less efficacious than PGAs, have more serious and a greater number of side effects and contraindications, and require multiple daily dosings. As a result, we believe there is a significant unmet need in both the PGA and non-PGA market segments, each of which represents approximately half of the U.S. and European glaucoma market based on prescription volumes. Despite the limitations of existing glaucoma drugs, Xalatan (latanoprost), the best-selling PGA, together with Xalacom, its fixed-combination with a beta blocker, which is not available in the United States, generated peak annual global revenues of approximately \$1.7 billion prior to the introduction of its generic equivalents, and the most commonly prescribed non-PGA drugs each generated peak annual global revenues of over \$400 million prior to the introduction of their generic equivalents.

We believe Rhopressa™ may be prescribed by eye-care professionals as an initial therapy for patients with low to moderately elevated baseline IOPs of 26 mmHg or below at the time of diagnosis, representing approximately 80% of glaucoma patients. At these IOP levels, we believe the amount of IOP reduction achieved by Rhopressa™ would be equal to or exceed that of all currently marketed PGA and non-PGA products.

In addition to the expected primary use of Rhopressa™ as an initial therapy for patients with low to moderately elevated baseline IOPs described above, we also believe Rhopressa™ may be prescribed by eye-care professionals in the following circumstances:

-

As an add-on drug of choice for patients taking PGAs, due to the MOAs of Rhopressa™ being complementary to the MOA of PGAs, and due to the strong efficacy, more convenient dosing and better tolerability profile of Rhopressa™ compared to currently marketed non-PGA add-on products. It is estimated that up to 50% of glaucoma patients receiving PGA monotherapy require add-on therapy within two years of initial prescription of the PGA in order to maintain control of IOP.

▲As a preferred alternative therapy for patients who do not respond to PGAs.

▲As a preferred initial therapy for patients with low-tension glaucoma.

Table of Contents

As a preferred initial therapy where PGAs are contraindicated and for patients who choose to avoid the cosmetic issues associated with PGAs, including iris color change in light-eyed patients, discoloration of tissue surrounding the eyes and eyelid droopiness and sunken eyes caused by loss of orbital fat.

In addition, based on our preclinical data to date, we believe that quadruple-action Roclatan™ would be the only glaucoma product that covers the full spectrum of currently known IOP-lowering MOAs, giving it the potential to provide a greater IOP-lowering effect than any currently approved glaucoma product. Therefore, we believe Roclatan™ could compete with both PGA and non-PGA therapies for patients requiring maximal IOP lowering, including those with IOPs above 26 mmHg and those who present with significant disease progression despite currently available therapies.

We own the worldwide rights to all indications for our current product candidates. We currently plan to commercialize our products ourselves in North America and possibly Europe and explore partnership opportunities through collaboration and licensing arrangements in certain key markets outside of North America, including Japan, emerging markets and possibly Europe. In Japan specifically, the Tajimi study found that 90% of glaucoma patients had IOP of 21 mmHg or below at the time of diagnosis. We believe this creates a significant market opportunity in Japan for Rhopressa™ due to its ability to reduce IOP at consistent levels across all tested baseline IOPs, as demonstrated in our Phase 2b clinical trial, which we believe differentiates it from currently marketed drugs that have shown reduced efficacy at lower baseline IOPs.

Our intellectual property portfolio contains patents and pending patent applications related to composition of matter, pharmaceutical compositions and methods of use for our product candidates. We have patent protection for our primary product candidates, Rhopressa™ and Roclatan™, in the United States through at least 2030.

Our Product Pipeline

Our primary product candidates, triple-action Rhopressa™ and quadruple-action Roclatan™, are once-daily eye drops. Rhopressa™ inhibits Rho Kinase, or ROCK, and the norepinephrine transporter, or NET, which are both novel biochemical targets for lowering IOP. By inhibiting these targets, we believe Rhopressa™ reduces IOP via three separate MOAs: (i) through ROCK inhibition, it increases fluid outflow through the TM, which accounts for approximately 80% of fluid drainage from the eye; (ii) also through ROCK inhibition, as demonstrated in a preclinical study, it reduces EVP, which represents the pressure of the blood in the episcleral veins of the eye where eye fluid drains into the bloodstream; and (iii) through NET inhibition, it reduces the production of eye fluid. Roclatan™, a single-drop fixed-dose combination of Rhopressa™ and latanoprost, lowers IOP through the same three MOAs as Rhopressa™ and, as a fourth MOA, through the ability of latanoprost to increase fluid outflow through the uveoscleral pathway, the eye's secondary drain.

We discovered and developed our product candidates internally through a rational drug design approach that coupled medicinal chemistry with high content screening of compounds in proprietary cell-based assays. We selected and formulated our product candidates for preclinical in vivo testing following a detailed characterization of over 1,500 synthesized ROCK-selective and ROCK/NET inhibitors. We continue to seek to discover and develop new compounds in our research laboratories and employ a scientific staff with expertise in medicinal chemistry, analytical chemistry, biochemistry, cell biology, pharmacology and pharmaceutical science.

The following table summarizes each of our existing product candidates, their MOAs and their development status, as well as our intellectual property rights for these product candidates.

Product Candidate and Mechanism	Phase of Development	Intellectual Property Rights
Rhopressa™ Triple-action—ROCK/NET inhibitor	Phase 3	Wholly-Owned
Roclatan™ Quadruple-action—ROCK/NET inhibitor and latanoprost, a PGA	Phase 3	Wholly-Owned

AR-13533

Second-generation ROCK/NET
inhibitor

Preclinical

Wholly-Owned

4

Table of Contents

Triple-Action Rhopressa™

Rhopressa™ is the first of a new class of glaucoma drug products that was discovered by our scientists. It is a once-daily eye drop designed to reduce IOP in patients with glaucoma or ocular hypertension. It increases fluid outflow through the primary drain of the eye while also reducing eye fluid production. In addition, a preclinical study demonstrated reduction of EVP as an additional MOA of Rhopressa™, as further described below. The active ingredient in Rhopressa™, AR-13324, acts through the inhibition of both ROCK and NET.

ROCK is a protein kinase, which is an enzyme that modifies other proteins by chemically adding phosphate groups to them. Specifically, ROCK regulates actin and myosin, which are proteins that are responsible for cellular contraction. ROCK activity also promotes the production of extracellular matrix proteins. ROCK inhibitors block TM cell contraction and reduce the production of extracellular matrix, thereby improving fluid outflow and consequently decreasing IOP. In addition, we believe ROCK inhibition may also be responsible for reduction of EVP. EVP represents the pressure of the blood in the episcleral veins of the eye, where eye fluid drains into the bloodstream. When EVP is lowered, the fluid is able to flow more freely from the eye.

NET is a protein that transports norepinephrine across neuronal cell membranes. Norepinephrine is a chemical released by neurons to communicate with targeted cells. NET returns excess norepinephrine back into the neuron, which helps end the signaling between the neuron and the neuron's target cells. We believe the inhibition of NET prolongs the activation of target cells in the ciliary body of the eye, which reduces the production of eye fluid and thereby lowers IOP.

In addition to its triple MOA, Rhopressa™ has a number of characteristics that distinguish it from our previously developed product candidates, including ROCK-selective drug AR-12286 and its fixed-dose combination product PG286, and other clinical-stage ROCK inhibitors, which together we refer to as "comparator ROCK inhibitors." The active ingredient in Rhopressa™, AR-13324, has a unique chemical composition that was specifically designed to allow maximal efficacy of the drug in the eye. Enzymatic conversion of AR-13324 produces two separate molecules, one of which is approximately ten to 160 times more potent at inhibiting ROCK than comparator ROCK inhibitors. This contributes to greater efficacy and longer duration of effect of AR-13324 relative to comparator ROCK inhibitors that we observed in preclinical models. In addition, AR-13324 has inhibitory activity against a secondary kinase target, Protein Kinase C, or PKC, which is known to act in parallel with ROCK to promote cell contraction. Compounds that inhibit ROCK without inhibiting PKC may allow PKC activity to increase in TM cells over time, resulting in a loss of IOP-lowering efficacy. We believe the ability of AR-13324 to inhibit both the primary, ROCK, and secondary, PKC, signaling pathways that lead to TM cell contraction contributes to the ability of Rhopressa™ to maintain its efficacy over time.

Rhopressa™ is expected to compete against all products in the glaucoma market, the significant majority of which have been in the market for over 20 years. The PGA and non-PGA market segments each represent approximately half of the U.S. and European glaucoma market based on prescription volumes. Despite the limitations of existing glaucoma drugs, Xalatan (latanoprost), the best-selling PGA, together with Xalacom, its fixed-combination with a beta blocker, which is not available in the United States, generated peak annual global revenues of approximately \$1.7 billion prior to the introduction of its generic equivalents, and the most commonly prescribed non-PGA drugs each generated peak annual global revenues of over \$400 million prior to the introduction of their generic equivalents. We believe there is a significant unmet need across the glaucoma market due to many drugs requiring multiple daily dosings, side effects and contraindications of other products, and the fact that none of the commonly prescribed drugs target the diseased TM tissue.

We believe that triple-action Rhopressa™ has several significant differentiating characteristics that would make it a strong competitor in both the PGA and non-PGA market segments, if approved, including:

Strong IOP-Lowering Effect-In our Phase 2b clinical trial, once-daily Rhopressa™ demonstrated mean IOP reductions of 5.7 and 6.2 mmHg on days 28 and 14, respectively. Studies have shown that a sustained 5 mmHg reduction in IOP reduces risk of disease progression by approximately 50%. In the Roclatan™ Phase 2b trial completed in June 2014, Rhopressa™ performed with similar results as it had in its Phase 2b trial completed in June 2013. Therefore, we believe

the level of IOP reduction achieved by Rhopressa™ would be equal to or exceed that of all currently marketed non-PGA products and, in addition, for patients with low to moderately elevated IOPs at the time of diagnosis, representing approximately 80% of glaucoma patients, would be equal to or potentially exceed that of all currently marketed PGA products.

Table of Contents

Consistent IOP-Lowering Effect Across Various Baseline IOPs-Published studies have indicated that currently marketed PGA and non-PGA products do not lower IOP as effectively in patients with low to moderately elevated baseline IOPs relative to patients with higher baseline IOPs. In our Phase 2b clinical trial, Rhopressa™ demonstrated a differentiated ability to reduce IOP at consistent levels across all baseline IOPs tested in the trial. The results of a preclinical in vivo study sponsored by Aerie and reported in February 2014 suggest that this differentiated effect may be attributable to the ability of Rhopressa™ to lower EVP.

Novel Triple-Action MOA-We believe Rhopressa™ works through three MOAs: increasing outflow through the TM, decreasing fluid production in the eye and reducing EVP. If approved, we believe Rhopressa™ would be the only once-daily drug available that works through these three MOAs. In addition, we believe the three MOAs of Rhopressa™ are highly complementary to the MOA of market-leading PGAs, which increase fluid outflow through the uveoscleral pathway.

Once-Daily Dosing Advantage-The most commonly prescribed non-PGA drugs are dosed two to three times daily, which places a considerable daily burden on patients, who are generally required to use these drugs for the remainder of their lives. Rhopressa™ is being developed as a once-daily dosed glaucoma therapy. This more convenient dosing regimen is expected to result in higher patient compliance, which may lead to improved outcomes.

Favorable Tolerability Profile-Currently marketed glaucoma drugs have several tolerability issues indicated on their product labels, including ocular allergic reaction, itching of the eye, iris color change, orbital tissue discoloration, unusual taste and hyperemia. In our Phase 2a and Phase 2b clinical trials for Rhopressa™, a total of 209 patients were exposed to Rhopressa™. The main tolerability finding for Rhopressa™ was transient, or temporary, hyperemia, which is a cosmetic asymptomatic redness of the eye. Most of the hyperemia was scored as “mild” as evaluated by the eye-care professionals in the morning following instillation of the drop the previous night. Hyperemia is a common tolerability finding also associated with PGAs. In the Roclatan™ Phase 2b trial completed in June 2014, Rhopressa™ tolerability findings were similar to those of the Phase 2b trial completed in June 2013.

Lack of Systemic Side Effects-Rhopressa™ has demonstrated a lack of systemic side effects in clinical trials to date, including our Phase 1 pharmacokinetic, or PK, study, the results of which were reported in January 2014. Currently marketed non-PGA drugs have systemic side effect issues indicated on their product labels, including among others, lethargy, reduced heart rate, Stevens Johnson syndrome and blood dyscrasias. Furthermore, the most widely prescribed non-PGA drug, timolol, has contraindications that include bronchospasm, arrhythmia and heart failure. In addition, Rhopressa™ targets the TM, the diseased tissue responsible for elevated IOP in glaucoma and the eye’s primary drain, whereas commonly prescribed PGAs and non-PGAs target the secondary drain and the fluid production in the eye, respectively.

Based on the Rhopressa™ Phase 2b clinical trial results, performance of Rhopressa™ in the Roclatan™ Phase 2b clinical trial and the several positive differentiating attributes of Rhopressa™, we believe Rhopressa™ has the potential to be a strong competitor across the glaucoma market. Our Phase 3 registration trials commenced in July 2014 and are designed to use timolol as the comparator, as timolol represents the most widely used comparator in registration trials in glaucoma, and is also the most widely prescribed non-PGA drug.

Rhopressa™ Phase 2b Efficacy Results

In May 2013, we completed a 28-day Rhopressa™ Phase 2b clinical trial. This trial included 221 patients who were treated once daily with Rhopressa™ 0.01%, Rhopressa™ 0.02% or latanoprost. Latanoprost was used as the comparator because it is the most widely prescribed drug of all currently marketed glaucoma products. The primary efficacy endpoint for this Phase 2b clinical trial was mean diurnal IOP across subjects within each treatment group on day 28. We observed statistically significant decreases in mean diurnal IOP in all treatment groups on day 28 as compared to unmedicated baseline.

Baseline IOP was measured prior to treatment. Following treatment, IOP was measured on day seven at 8 a.m. and on days 14 and 28 at 8 a.m., 10 a.m. and 4 p.m. On day 14, mean diurnal IOP (which refers to the average of mean IOPs measured at 8 a.m., 10 a.m. and 4 p.m.) decreased to 19.5 and 18.4 mmHg in the Rhopressa™ 0.02% and latanoprost groups, respectively,

Table of Contents

representing a decrease from unmedicated baseline of 6.2 and 7.1 mmHg. On day 28, mean diurnal IOP was 20.0 and 18.7 mmHg, respectively, representing a decrease from unmedicated baseline of 5.7 and 6.8 mmHg. These decreases from unmedicated baseline were statistically significant with p-values < 0.001. P-value, or probability value, is a statistical measure that helps scientists determine if their hypotheses are correct. It is directly related to the statistical significance level of the results, which is an important component in determining whether the data obtained from scientific research support the hypothesis being tested.

The statistical significance level is determined by the researcher and is customarily set at 0.05, or 5%. Essentially, this means that 5% of the time, the results in the study would be derived by complete chance, but 95% of the time, the variable in the study would be directly related to the results of the study. Efficacy from the Phase 2b clinical trial are described further below.

Efficacy Results of the 28-day Phase 2b Clinical Trial Comparing Rhopressa™ 0.02% to Latanoprost Showing Mean Diurnal IOP for Days 14 and 28 Compared to Baseline

Rhopressa™ maintained consistent efficacy from day seven to day 28. For Rhopressa™ 0.02%, the concentration being used in our Phase 3 trials, at the 8 a.m. time point, the time of highest baseline IOP, the IOP reductions achieved on day seven and day 28 were 6.0 and 5.9 mmHg, respectively. The level of IOP reduction achieved by Rhopressa™ 0.02% in our Phase 2b study was clinically significant, since previously published long-term studies have demonstrated that a sustained 5 mmHg reduction in IOP reduces the risk of disease progression by approximately 50%.

“Clinical significance” means that the effect is large enough to be important to patients and physicians. An effect that is statistically significant may or may not also be clinically significant. In glaucoma, the Early Manifest Glaucoma Trial, a large long-term study evaluating the effect of IOP lowering in patients with glaucoma, concluded that each 1 mmHg reduction in IOP lowered the risk of progression of optic nerve damage by 10%, indicating that each 1 mmHg reduction in IOP provides a meaningful level of protection to the patient.

IOP-Lowering Effect of Rhopressa™ 0.02% at 8 a.m. on Days 7, 14 and 28

Table of Contents

In the full Phase 2b trial population, which consisted of patients with unmedicated baseline IOPs ranging from 22 to 36 mmHg, the IOP-lowering effect of our once-daily Rhopressa™ 0.02% was 1.2 mmHg less than that of latanoprost on day 28 and did not show non-inferiority. However, Rhopressa™ 0.02% efficacy relative to latanoprost was in line with published historical data for twice-daily timolol relative to latanoprost. Timolol is the most commonly prescribed non-PGA drug and the comparator for our Phase 3 non-inferiority registration trials.

A study by Hedman and Alm, which reports on the pooled data from three registration trials of latanoprost versus timolol, showed the IOP-lowering effect of timolol to be 1.2 mmHg less than that of latanoprost, as reflected in the graph on the following page under the heading “Comparison of Latanoprost and Timolol from Pooled Data of Three Registration Trials.” Our Rhopressa™ Phase 2b clinical trials similarly showed Rhopressa to have an IOP-lowering effect of 1.2 mmHg less than that of latanoprost.

An additional protocol-specified analysis that compared the results for the patients who entered the trial with moderately elevated baseline IOPs (22 to 26 mmHg) to patients with highly elevated baseline IOPs (greater than 26 mmHg) revealed a differentiated efficacy profile of Rhopressa™ compared to latanoprost. Consistent with previous scientific literature, latanoprost produced smaller IOP reductions in patients with moderately elevated IOPs than in patients with highly elevated IOPs. In contrast, Rhopressa™ maintained essentially the same IOP-lowering effect in patients with moderately elevated IOPs as in patients with highly elevated IOPs ($p>0.30$). As a result, the IOP-lowering effect of Rhopressa™ was equivalent to latanoprost in patients with moderately elevated baseline IOPs and Rhopressa™ thereby demonstrated statistical non-inferiority to latanoprost in this sub-group. A non-inferiority trial is a type of clinical trial performed to see if a new drug or treatment is “not inferior” to a current active treatment or to determine if a new treatment is “at least as good as,” or “not unacceptably worse than,” the active comparator treatment. A non-inferiority trial aims at demonstrating that the test product is not worse than the comparator by more than a small pre-specified amount. This amount is known as the non-inferiority margin, which for the Rhopressa™ Phase 2b trial was 1.5 mmHg.

IOP-Lowering Effect of Rhopressa™ 0.02% and Latanoprost in the Full Patient Population
Compared to the Subgroup with Moderately Elevated IOP*

* Based on diurnal measurements.

A study published in 2000, which pooled data from three latanoprost registration trials, demonstrated that both latanoprost and timolol lose approximately 0.5 mmHg in efficacy for every 1 mmHg lower baseline IOP, as illustrated in the chart below. Additional publications have indicated similar declining efficacy results for other currently marketed non-PGA glaucoma drugs, including the alpha agonist brimonidine and the carbonic anhydrase inhibitor dorzolamide.

Table of Contents

Comparison of Latanoprost and Timolol from Pooled Data of Three Registration Trials

Source: Hedman and Alm (Eur J Ophthalmol 2000; 10:95-104)

We believe the ability of Rhopressa™ to maintain a consistent IOP-lowering effect on baseline IOP will place Rhopressa™ in a favorable competitive position relative to current PGA and non-PGA products because a significant majority of glaucoma patients have baseline IOPs of 26 mmHg or below at the time of diagnosis. Results from a large epidemiological survey published in 1991, the Baltimore Eye Survey, demonstrated that greater than 78% of patients have unmedicated baseline IOPs of 26 mmHg or below when first diagnosed with glaucoma.

Prevalence of Glaucoma by Baseline IOP at the Time of Diagnosis

Adapted from Baltimore Eye Survey in which 10,444 subjects were screened for the prevalence of Open-Angle Glaucoma (OAG)

Furthermore, in the Tajimi Study carried out in Japan in 2000 and 2001, 92% of patients with primary open-angle glaucoma were found to have IOPs of 21 mmHg or less at the time of diagnosis. In this study, 3,870 randomly selected residents of the city of Tajimi were screened for primary open-angle glaucoma.

Table of Contents

Rhopressa™ Phase 2a Efficacy Results

In August 2012, we completed a 7-day Rhopressa™ Phase 2a clinical trial. This trial included 85 patients who were treated once-daily with Rhopressa™ 0.01%, Rhopressa™ 0.02%, Rhopressa™ 0.04% or the vehicle of Rhopressa™. “Vehicle” refers to the formulation without the active ingredient. Baseline IOP was measured prior to treatment. IOP was measured following seven days of dosing at 8 a.m., 10 a.m., 12 p.m. and 4 p.m. The primary efficacy endpoint for this Phase 2a clinical trial was the mean diurnal IOP (which refers to the average of mean IOPs measured at 8 a.m., 10 a.m., 12 p.m. and 4 p.m.) across subjects within each treatment group on day eight. We observed statistically significant decreases in mean diurnal IOP in all Rhopressa™ treatment groups following seven days of dosing compared to unmedicated baseline. Additionally, each concentration of Rhopressa™ was shown to be statistically superior to the vehicle following seven days of dosing with p-values ranging from 0.018 to <0.001.

Rhopressa™ Phase 2 Safety Data

In our 7-day Phase 2a and 28-day Phase 2b clinical trials for Rhopressa™ and Roclatan™ a total of 287 patients were exposed to Rhopressa™. In these trials, Rhopressa™ was well tolerated. The main adverse event was transient hyperemia, or asymptomatic redness of the eye, with all hyperemia scored as mild or moderate. This cosmetic tolerability finding is based on the MOA of the drug, which induces a transient dilation of small blood vessels located over the sclera, or white part of the eye.

The biomicroscopy findings for the vast majority of patients who experienced ocular hyperemia in the Rhopressa™ Phase 2b trial were mild and transient, and there were no observations of severe ocular hyperemia. Biomicroscopy refers to the observation by a masked examiner of the anterior part of the eye. On day 28 at 8 a.m., mild and moderate conjunctival hyperemia was observed in 18% and 24% of patients in the Rhopressa™ 0.01% and 0.02% treatment groups, respectively, and in 11% of patients in the latanoprost group. The incidence of conjunctival hyperemia decreased throughout the study for Rhopressa™ and increased for latanoprost.

Published data indicate that latanoprost generates the lowest rate of hyperemia among the commonly prescribed PGAs. In a study that compared the relative frequency of hyperemia for bimatoprost, travaprost and latanoprost after 12 weeks of treatment, the largest proportion of patients reporting redness was found in the bimatoprost group with 35%, followed by the travaprost and latanoprost groups with 27% and 16%, respectively.

Rhopressa™ Comparison to AR-12286

We have analyzed our clinical and preclinical data for Rhopressa™, the lead candidate from our ROCK/NET inhibitor class, relative to our clinical and preclinical data for AR-12286, our ROCK-selective compound that we were previously evaluating for further clinical development in addition to Rhopressa™. We conducted similarly designed 28-day Phase 2 clinical trials for each of Rhopressa™ and AR-12286, the comparative results of which are presented in the chart below. Rhopressa™ 0.02% maintained stable efficacy on day 28 relative to day seven in its 28-day Phase 2 clinical trial. In contrast, AR-12286 0.5% lost 1.4 mmHg of IOP-lowering efficacy from day seven to day 28 in its 28-day Phase 2 clinical trial.

IOP-Lowering Effect of Rhopressa™ and AR-12286
at 8 a.m. on Days 7, 14 and 28

Table of Contents

We subsequently completed a three-month Phase 2 clinical trial for AR-12286, for which data were available in June 2013. This trial confirmed the trend observed in the 28-day trial discussed above. In the three-month trial, the efficacy of AR-12286 continued to decline over the trial period such that it failed to meet its primary efficacy endpoint, non-inferiority to timolol.

Our lead product candidate, Rhopressa™, has a number of characteristics that distinguish it from AR-12286. Rhopressa™ lowers IOP by inhibiting both ROCK and NET, whereas AR-12286 inhibits only ROCK. In addition, the active ingredient in Rhopressa™, AR-13324, has a unique chemical composition that was specifically designed to allow maximal efficacy of the drug in the eye. Enzymatic conversion of AR-13324 produces two separate molecules, one of which is approximately ten times more potent at inhibiting ROCK than AR-12286. The more potent ROCK inhibition provided by Rhopressa™, as well as its ability to inhibit NET, contributes to its greater efficacy and longer duration of effect relative to AR-12286.

In addition, the analyses of our data suggest that there is a secondary signaling pathway that is activated by a protein called PKC that also leads to contraction of the TM. Our preclinical analyses show that AR-13324 is a potent inhibitor of both ROCK and PKC, whereas AR-12286 is a potent inhibitor of ROCK but not of PKC. We believe that the ability of AR-13324 to inhibit both the primary, ROCK, and the secondary, PKC, signaling pathways that lead to TM cell contraction contributes to the ability of Rhopressa™ to maintain its efficacy over time.

The chart below shows the duration of effect of Rhopressa™ 0.02% from our 28-day Phase 2b clinical trials for Rhopressa™ and Roclatan™ as compared with latanoprost, which we believe has a longer duration of effect than AR-12286. In both the Phase 2b clinical trials for Rhopressa™ and Roclatan™, Rhopressa™ 0.02% had superior duration 36 hours after last dosing relative to latanoprost.

Furthermore, in a six-month toxicology study with exaggerated dosing of AR-12286, lens opacities, otherwise known as cataracts, were observed in rabbit eyes beginning at three months. In a similar six-month toxicology study with exaggerated dosing of Rhopressa™, no adverse lens effects were observed.

As a result of these observations, in June 2013, we selected Rhopressa™ for advancement to Phase 3 clinical development and discontinued development of AR-12286 and its related fixed-dose combination product PG286.

Rhopressa™ Phase 1 Pharmacokinetic Study Results

In January 2014, we reported top-line results from our Phase 1 PK study, in which Rhopressa™ eye drops were administered once daily to 18 healthy individuals over an eight-day period to assess systemic exposure to the drug. In addition, the drug's effect on IOP was measured. All study subjects had normotensive IOPs in the range of 12 to 21 mmHg, with an average diurnal IOP for the group of approximately 16 mmHg prior to dosing. The PK study demonstrated very low systemic exposure to Rhopressa™, with blood levels at or below the limit of detection of 0.1 ng/mL at all time points, and no drug-related effects on systemic safety parameters such as blood pressure and heart rate. Of particular importance to the product's efficacy profile, the subjects' average diurnal IOP decreased by approximately 5 mmHg, or more than 30%, to approximately 11 mmHg after the eight days of dosing. We believe this large IOP reduction in normotensive subjects was due to the EVP-lowering effect of Rhopressa™, which has been subsequently supported by a preclinical in vivo study described below.

Table of Contents**Rhopressa™ Preclinical in Vivo Study Results**

We believe that the strong IOP-lowering effect of Rhopressa™ at lower baseline IOPs, and its consistent IOP-lowering effect across all tested baseline IOPs, are due in part to the ability of Rhopressa™ to lower EVP, which accounts for approximately half of IOP in normotensive individuals. This is an MOA that we believe further differentiates Rhopressa™ from currently marketed PGA and non-PGA products. The EVP-lowering effect of Rhopressa™ was demonstrated in a preclinical in vivo rabbit study sponsored by Aerie, the results of which we reported in February 2014. In this study, Rhopressa™ demonstrated statistically significant reductions in EVP and IOP following the third daily dose. EVP decreased by 35% relative to baseline, and IOP was reduced by 39%. Based on these study results, it was estimated that up to 42% of the reduction in IOP caused by Rhopressa™ was due to the reduction in EVP.

Rhopressa™ Development Strategy

Phase 3 registration trials for Rhopressa™ commenced in July 2014. We anticipate total enrollment of approximately 1,300 patients in three Phase 3 registration trials of Rhopressa™. Phase 3 efficacy results will be determined after three months of treatment and safety results will be analyzed and submitted following 12 months of treatment. Two trials are being conducted in the United States, named “Rocket 1” and “Rocket 2,” and one safety-only study is being conducted in Canada, named “Rocket 3.”

The entry criteria for our Phase 3 trials include a minimum IOP greater than 20 mmHg and a maximum of less than 27 mmHg. Based on discussions with the FDA, we believe that the entry criteria for our Phase 3 trials will not impact the product label. The entry criteria for our Phase 2 trials were 22 to 36 mmHg. Lowering the IOP entry criteria for our Phase 3 trials increases the representation of patients with moderately elevated IOPs in the trials and thereby provides a more representative cross-section of the glaucoma patient population. The primary efficacy endpoint of the trials will be to demonstrate non-inferiority of IOP lowering for Rhopressa™ compared to timolol. Timolol is the most widely used comparator in registration trials for glaucoma and also the most widely prescribed non-PGA glaucoma drug.

Pending successful advancement of the Phase 3 registration trials, three-month efficacy results are expected in the middle of the second quarter 2015 for Rocket 1 and in mid-2015 for Rocket 2. If the results of the Phase 3 trials are positive, then we would submit a new drug application, or an NDA, by mid-2016. We intend to explore the potential for priority review with the FDA, although there can be no assurance that such priority review will be granted by the FDA.

Quadruple-Action Roclatan™

Our once-daily, quadruple-action product candidate Roclatan™ is a combination of our triple-action compound AR-13324, the active ingredient in Rhopressa™, formulated with latanoprost in a single eye drop. If approved, we believe that Roclatan™ would be the first glaucoma product to lower IOP through all currently known MOAs:

- increasing fluid outflow through the TM, the eye’s primary drain,
- reducing fluid production in the eye,
- reducing EVP, and
- through the MOA of latanoprost, increasing fluid outflow through the uveoscleral pathway, the eye’s secondary drain.

Roclatan™ Phase 2 Efficacy Results

In June 2014, we completed a 28-day Roclatan™ Phase 2b clinical trial. The baseline IOPs tested in the study ranged from 22 to 36 mmHg and included 297 patients who were treated once daily with Roclatan™ 0.01%, Roclatan™ 0.02%, Rhopressa™ 0.02%, or latanoprost. The primary efficacy endpoint for this Phase 2b clinical trial was statistical superiority of Roclatan™ over each of its components on day 29. Baseline IOP was measured prior to treatment. Following treatment, IOP was measured on day eight, 14 and 28 at 8 a.m., 10 a.m. and 4 p.m. We observed statistical superiority over the individual components at all time points.

Table of Contents

Roclatan™ vs. Individual Components
Mean IOP at All Time Points (p<0.001)

Roclatan™ 0.02% lowered mean diurnal IOP on day 29 from 25.1 mmHg at baseline to 16.5 mmHg, a 34% decrease in IOP. Roclatan™ 0.02% was determined to be 1.6 - 3.2 mmHg more efficacious than latanoprost and 1.7 - 3.4 mmHg more efficacious than Rhopressa™.

Roclatan™ Efficacy vs. Individual Components
Mean IOP at All Time Points (Intent to Treat)

* Difference between 0.02% Roclatan™ and latanoprost or Rhopressa™

An additional analysis that compared the response results for patients on day 29 revealed that 50% of Roclatan™ patients compared to 28% of latanoprost patients experienced a 35% or greater decrease in mean diurnal IOP from baseline on day 29. Furthermore, 46% of Roclatan™ patients compared to 18% of latanoprost patients had a mean diurnal IOP of 16 mmHg or less on day 29.

Table of Contents

We believe Roclatan™, if approved, would be the only glaucoma product that covers the full spectrum of currently known IOP-lowering MOAs, giving it the potential to provide a greater IOP-lowering effect than any currently marketed glaucoma product. Therefore, we believe Roclatan™ could compete with both PGA and non-PGA therapies for patients requiring maximal IOP lowering, including those with IOPs above 26 mmHg and those who present with significant disease progression despite currently available therapies.

Roclatan™ Phase 2 Safety Data

In our Phase 2b clinical trial, a total of 147 patients were exposed to Roclatan™. In these trials, Roclatan™ was well tolerated. The most common Roclatan™ adverse event was hyperemia, or eye redness, which was reported in 40% of patients. For patients who experienced hyperemia, 80% were observed as mild through biomicroscopy findings. Additionally, there were no systemic drug-related adverse events reported.

Roclatan™ Development Strategy

Registration trials for Roclatan™ are expected to commence in mid-2015 upon completion of our three-month Phase 3-enabling ocular toxicology study and ocular PK study. Additionally, we expect to have further discussions with the FDA and European Medicines Agency (“EMA”) regarding final trial designs in the first quarter of 2015.

We currently anticipate total enrollment of approximately 1,500 patients in three Phase 3 registration trials of Roclatan™. We expect there will be two trials conducted in the United States and one trial in the European Union. As described above, we intend to meet with the FDA and EMA in the first quarter of 2015 regarding final trial designs. As such, there can be no assurance that our anticipated enrollment and trial designs described above will satisfy both the FDA and EMA and subsequent adjustments to the trial designs may be necessary.

Assuming we commence the Phase 3 trials in mid-2015 and fully enroll the trials within our anticipated timeframe, we would expect efficacy data from the trials in mid-2016 and, if the results of the Phase 3 trials are positive, that we would submit a NDA by mid-2017. We intend to explore the potential for priority review with the FDA, although there can be no assurance that such priority review will be granted by the FDA.

Second-Generation AR-13533

In addition to our primary product candidates, Rhopressa™ and Roclatan™, we are in the preclinical development stage with AR-13533, our second-generation ROCK/NET inhibitor. AR-13533 does not require enzymatic conversion in the eye to deliver maximal ROCK inhibitor activity, and therefore AR-13533 may provide additional IOP-lowering effect in patients beyond that obtained with Rhopressa™. We have not submitted an IND for AR-13533 to the FDA and there can be no assurance that an IND will be submitted.

Our Strategy

Our goal is to be a leader in the discovery, development and commercialization of innovative pharmaceutical products for the treatment of patients with glaucoma and other diseases of the eye. We believe our product candidates have the potential to address many of the unmet medical needs in the glaucoma market. Key elements of our strategy are to: Advance the development of our product candidates to approval. Based on the results from our Phase 2b clinical trial for triple-action Rhopressa™, we proceeded into Phase 3 registration trials for this drug in July 2014. In June 2014, we successfully completed a Phase 2b clinical trial for Roclatan™, our quadruple-action combination of Rhopressa™ and latanoprost, and preparatory steps for Phase 3 registration trials have commenced. We expect Phase 3 registration trials for Roclatan™ to commence in mid-2015. In addition, over the longer term, we plan to evaluate opportunities associated with preclinical-stage AR-13533, our second-generation ROCK/NET inhibitor.

Establish internal sales capabilities to commercialize our product candidates in North America and possibly Europe. We own worldwide rights to all indications for our product candidates and we plan to retain North American and possibly European commercialization rights. Ultimately, if our product candidates are approved, we plan to build a commercial team of approximately 100 sales representatives. We expect our sales organization to target approximately 10,000 high prescribing eye-care professionals throughout North America. If our product candidates are approved in Europe for commercial sale and if we self-commercialize our product candidates in Europe, we will need to establish similar functions or outsource these functions to third parties.

Table of Contents

Explore partnerships with leading pharmaceutical and biotechnology companies to maximize the value of our product candidates outside North America. We currently plan to explore the licensing of commercialization rights or other forms of collaboration with qualified potential partners for the commercialization of our product candidates in other territories, including Japan and possibly Europe.

Continue to leverage and strengthen our intellectual property portfolio. We believe we have a strong intellectual property position relating to our product candidates. Our intellectual property portfolio contains patents and pending patent applications in the U.S. and certain foreign jurisdictions related to composition of matter, pharmaceutical compositions and methods of use for our product candidates. We have patent protection for our primary product candidates in the United States through at least 2030.

Expand our product portfolio through internal discovery efforts and possible in-licensing or acquisitions of additional ophthalmic product candidates or products. We continue to seek to discover and develop new compounds in our research laboratories and employ a scientific staff with expertise in medicinal chemistry, analytical chemistry, biochemistry, cell biology, pharmacology and pharmaceutical science. In addition, we also plan to evaluate the expansion of our product portfolio through in-licensing or acquisitions of additional ophthalmic product candidates or products.

Glaucoma Overview

Glaucoma is generally characterized by relatively high IOP as a result of impaired drainage of fluid, known as aqueous humor, from the eye. The FDA recognizes sustained lowering of IOP, measured in terms of mmHg, as the primary clinical endpoint for regulatory approval, making clinical trials for this indication relatively straight-forward due to easily measured objective parameters.

In a healthy eye, aqueous humor is continuously produced and drained from the eye in order to maintain pressure equilibrium and provide micronutrients to various tissues in the eye. The normal range of IOP is generally between 10 and 21 mmHg. Several studies have demonstrated that the significant majority of glaucoma patients have IOPs below 26 mmHg at the time of diagnosis. An insufficient drainage of fluid can increase IOP above normal levels, which can eventually cause damage to the optic nerve. Once damaged, the optic nerve cannot regenerate and thus, damage to vision is permanent.

The most common form of glaucoma is open-angle glaucoma, which is characterized by abnormally high IOP as a result of impaired drainage of fluid from the eye's primary drain, the TM. Open-angle glaucoma is a progressive disease leading to vision loss and blindness for some patients as a result of irreversible damage to the optic nerve. Studies of the disease have demonstrated that reducing IOP in patients with glaucoma can help slow or halt further damage to the optic nerve and help preserve vision. Once diagnosed, glaucoma requires life-long treatment to maintain IOP at lower levels based on the individual patient's risk of disease progression. Ophthalmologists will routinely determine a target IOP, which represents the desired IOP level to achieve with glaucoma therapy for an individual patient. Should the disease progress even once the initial target IOP is reached, further lowering of the IOP has been shown to help in preventing additional damage to the optic nerve and further vision loss. This may require lowering IOP until it is in the so-called "low normal range" of 12 to 14 mmHg to protect the optic nerve from further damage.

There are multiple factors that can contribute to an individual getting open-angle glaucoma, including age, family history and ethnicity. For example, there generally is a higher incidence and severity of the disease in African-American and Hispanic populations.

Some patients with high IOP are diagnosed with a condition known as ocular hypertension. Patients with ocular hypertension have high IOP without the loss of visual fields or observable damage to the optic nerve, and are at an increased risk of developing glaucoma. These patients are commonly treated in the same manner as glaucoma patients.

Table of Contents

The following diagram illustrates how increased IOP eventually leads to increased pressure on the optic nerve, resulting in gradual loss of vision and ultimately visual disability and blindness.

The ciliary body in the eye is the tissue that produces aqueous humor, the production of which is commonly referred to as fluid inflow. The fluid leaves the eye primarily through the TM, the process of which is commonly referred to as fluid outflow. The healthy eye maintains a state of IOP homeostasis through a constant physiological process of aqueous humor production and drainage. The deteriorating function of the TM in glaucoma leads to increased resistance to fluid outflow and higher IOP. There is also a secondary drain for the fluid in the eye known as the uveoscleral pathway, which is typically responsible for approximately 20% of fluid drainage.

In addition to aqueous humor production and drainage through the TM and uveoscleral pathway, EVP plays a significant role in the regulation of IOP. EVP represents the pressure of the blood in the episcleral veins of the eye which are the site of drainage of eye fluid into the bloodstream. Historical studies have shown that EVP accounts for approximately half of IOP in normotensive subjects and approximately one-third of IOP in patients with pressures of 24 to 30 mmHg. When EVP is lowered, aqueous humor is able to flow more freely from the eye.

Patients are diagnosed through measurements of IOP using Goldmann applanation tonometry, the standard device used by clinicians to measure IOP, along with an evaluation of visual fields and observing the appearance of the optic nerve. These tests are routinely carried out by eye-care professionals. The initial treatment for patients diagnosed with open-angle glaucoma or ocular hypertension is typically a PGA eye drop. PGAs are designed to lower IOP by increasing outflow through the eye's secondary fluid drain. An eye-care professional will then measure a patient's response to the drug over the first few months. It has been shown that up to 50% of glaucoma patients require more than one drug to treat their IOP. This may occur as early as three to six months after initiating treatment with a PGA. The eye-care professionals may then add a second drug from one of the non-PGA classes, to be used together with the initial drug, or switch to a fixed-combination of two drugs in a single eye drop, or select an alternative single treatment. The reason so many patients eventually need more than one drug is generally considered to be a reflection of the progressive nature of the disease at the TM.

In severe glaucoma cases, patients may need to undergo an invasive surgical procedure. Trabeculectomy is the most common glaucoma-related surgical procedure, also referred to as filtration surgery, in which a piece of tissue in the drainage angle of the eye is removed, creating an opening to the outside of the eye. The opening is partially covered with a scleral flap, the white part of the eye, and the conjunctiva, the thin membrane covering the sclera. This new opening allows fluid to drain out of the eye, bypassing the clogged drainage channels of the TM to maintain a lowered IOP. Devices called shunts are used in glaucoma surgery to divert fluid in a controlled manner from the inside of the eye to the subconjunctival space bypassing the blocked TM. Generally, the shunts reduce IOP to the extent that the use of drops can be reduced, but often not completely eliminated. Many patients continue to require eye drops even following surgery.

Competition

The pharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our experience and scientific knowledge provide us with competitive advantages, we face competition from established branded and generic pharmaceutical companies, such as Bausch + Lomb, Inc. (acquired in 2013 by Valeant Pharmaceuticals International, Inc.), Merck & Co., Inc., Novartis International AG, Allergan, Inc. (acquired in 2014 by Actavis plc), Santen Inc. and smaller biotechnology and pharmaceutical companies as well as from academic institutions, government agencies and private and public research institutions, which may in the future develop products to treat

Table of Contents

glaucoma. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. We believe that the key competitive factors affecting the success of our product candidates, if approved, are likely to be efficacy, safety, convenience, price, tolerability and the availability of reimbursement from government and other third-party payors.

We expect to compete directly against companies producing existing and future glaucoma treatment products. The most commonly approved classes of eye drops to lower IOP in glaucoma are discussed below:

PGA Drug Class

Prostaglandin Analogues (PGAs). Most PGAs are once-daily dosed eye drops generally prescribed as the initial drug to reduce IOP by increasing fluid outflow through the eye's secondary drain. They do not target the diseased tissue, or TM. PGAs represent approximately half of the U.S. and European prescription volume for the treatment of glaucoma.

Xalatan (latanoprost), the best-selling PGA, together with Xalacom, its fixed-combination with a beta blocker, which is not available in the United States, had worldwide peak sales of approximately \$1.7 billion before its patent expired in 2012, according to publicly reported sales. The adverse effects of PGAs include hyperemia or eye redness, irreversible change in iris color, discoloration of the skin around the eyes, and droopiness of eyelids caused by the loss of orbital fat. PGAs should be used with caution in patients with a history of intraocular inflammation.

Non-PGA Drug Class

Beta Blockers. Beta blockers, with their MOA designed to inhibit aqueous production, are one of the oldest approved drugs for the lowering of IOP. The most commonly used drug in this class is timolol. Beta blockers are less effective than PGAs in terms of IOP lowering and are typically used twice daily. Beta blockers are the most commonly used non-PGA drug. They are used as an initially prescribed monotherapy and as an adjunct therapy to PGAs when the efficacy of PGAs is insufficient. Beta blocker eye drops have contraindications in their label as a result of systemic exposure from topical application of the eye drops, potentially leading to cardio-pulmonary events such as bronchospasm, arrhythmia and heart failure.

Carbonic Anhydrase Inhibitors. Carbonic anhydrase inhibitors, with their MOA designed to inhibit aqueous production, are less effective than PGAs and are required to be dosed three times daily in order to obtain the desired IOP lowering. In published clinical studies of carbonic anhydrase inhibitors, the most frequently reported adverse events reported were blurred vision and bitter, sour or unusual taste. Carbonic anhydrase inhibitors are sulfonamides and, as such, systemic exposure increases risk of adverse responses such as Stevens Johnson syndrome and blood dyscrasias.

Alpha Agonists. Alpha agonists, with their MOA designed to inhibit aqueous production plus have an effect on uveoscleral outflow, are less effective than PGAs and need to be dosed three times daily in order to obtain the desired IOP lowering. In clinical studies, the most frequently reported adverse reactions that occurred in individuals receiving brimonidine ophthalmic solution, a commonly prescribed alpha agonist, included allergic conjunctivitis, conjunctival hyperemia, eye pruritus, burning sensation, conjunctival folliculosis, hypertension, ocular allergic reaction, oral dryness and visual disturbance.

Despite their modest efficacy, safety and tolerability profiles, the requirement for two to three doses per day, and the fact that they do not target the diseased tissue in glaucoma, the beta blocker, carbonic anhydrase inhibitor and alpha agonist products account for up to half of the total prescription volume for the treatment of glaucoma based on historical prescription patterns, with beta blocker timolol being the most widely prescribed non-PGA drug. This is driven by the PGA products not being sufficiently effective as monotherapy for up to half of all glaucoma patients. Among the non-PGA drug classes, brands such as Allergan's Alphagan / Combigan franchise generated combined global revenues in 2012 of over \$420 million, and prior to the introduction of generics, the branded beta blockers and carbonic anhydrase inhibitors generated peak annual product revenues of over \$400 million. Despite targeting the secondary drain and not the diseased TM, and despite cosmetic side effects, Xalatan (latanoprost), the best-selling PGA, and Xalacom, its fixed-combination with a beta blocker, which is not available in the United States, generated

peak annual global revenues of approximately \$1.7 billion prior to the introduction of its generic equivalents. Fixed-combination glaucoma products are currently marketed in the United States, including Cosopt, the combination of a beta blocker with a carbonic anhydrase inhibitor, and Combigan, the combination of a beta blocker with an alpha agonist. In April 2013, Alcon announced FDA approval of Simbrinza, a fixed-dose combination of brinzolamide, a carbonic anhydrase inhibitor, and brimonidine tartrate, an alpha agonist, which requires dosing three times per day. There are no fixed-combinations of PGAs with other glaucoma drugs currently available in the United States.

Table of Contents

In addition to demonstrating suboptimal efficacy and safety profiles, many of the older glaucoma drugs are associated with compliance issues. For example, non-compliance can result from the difficulty of administering multiple eye drops in a single day. Challenges such as this are magnified for elderly patients, who constitute a large and growing proportion of the glaucoma population.

Administering multiple eye drops two or three times daily also increases exposure of patients to the preservatives in eye drops. Over time, this increased exposure may lead to damage to the surface of the cornea resulting in discomfort and symptoms of dry eye disease.

New eye drops for the treatment of glaucoma continue to be developed by our competitors. The following table outlines publicly disclosed development programs for the treatment of glaucoma of which we are aware.

New MOAs

Brand	MOA / Dosing	Trial Stage
Rhopressa™ (Aerie AR-13324)	ROCK/NET inhibitor (qd)	Phase 3
Roclatan™ (Aerie PG324)	ROCK/NET inhibitor + PGA (qd)	Phase 3
K-115 (Kowa)	ROCK inhibitor (bid)	Approved in Japan ¹
AMA0076 (Amakem)	ROCK inhibitor (bid)	Phase 2a
INO-8875 (Inotek)	Adenosine-A1 agonist (bid or qd)	Phase 2
OPA-6566 (Acucela)	Adenosine-A2a agonist (bid)	Phase 1/2
SYL040012 (Sylentis)	RNAi beta blocker (qd)	Phase 2

New PGAs²

Brand	MOA / Dosing	Trial Stage
BOL-303259 (Bausch + Lomb)	NO donating latanoprost (qd)	Phase 3
DE-117 (Santen)	EP2 agonist (qd)	Phase 2
ONO-9054 (Ono)	FP/EP3 agonist (qd)	Phase 2

¹Approved in Japan on September 29, 2014 as an adjunctive therapy.

²Not usable as add-on therapy to current PGAs.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. In early 2013, Sucampo Pharmaceuticals, Inc. commercially relaunched Rescula, a twice-daily dosed PGA, with the claim that it reduces elevated IOP by increasing the outflow of aqueous humor through the TM. Additionally, Bausch + Lomb Inc., a wholly owned subsidiary of Valeant Pharmaceuticals International, Inc., is developing a nitric oxide-donating latanoprost and is currently in Phase 3 clinical trials. Early-stage companies are also developing glaucoma treatments and may prove to be significant competitors, such as Inotek Pharmaceuticals, which is developing an adenosine receptor agonist. We expect that our competitors will continue to develop new glaucoma treatments, which may include eye drops, oral treatments, surgical procedures, implantable devices or laser treatments. Alternative treatments beyond eye drops continue to develop.

Other early-stage companies may also compete through collaborative arrangements with large and established companies. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer adverse effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. In addition, our ability to compete may be affected because in many cases insurers or other third-party payors encourage the use of generic products. Our industry is highly competitive

and is currently dominated by generic drugs, such as latanoprost and timolol, and additional products are expected to become available on a generic basis over the coming years. If any of our product candidates are approved, we expect that they will be priced at a premium over competitive generic products and consistent with other branded glaucoma drugs.

18

Table of Contents

Manufacturing

AR-13324, the active ingredient in Rhopressa™, is a small molecule and capable of being manufactured in reliable and reproducible synthetic processes from readily available starting materials. We believe the chemistry used to manufacture AR-13324 and Rhopressa™ is amenable to scale up and does not require unusual equipment in the manufacturing process. We do not currently operate manufacturing facilities for clinical or commercial production of our product candidates. We currently rely on third-party manufacturers to produce the active pharmaceutical ingredient and final drug product for our clinical trials. We manage such production with all our vendors on a purchase order basis in accordance with applicable master service and supply agreements. We do not have long-term agreements with any of these or any other third-party suppliers to support our clinical trials. Latanoprost, used in the manufacture of Roclatan™, is available in commercial quantities from multiple reputable third-party manufacturers. We intend to procure quantities on a purchase order basis for our clinical and commercial production. If any of our existing third-party suppliers should become unavailable to us for any reason, we believe that there are a number of potential replacements, although we might experience a delay in our ability to obtain alternative suppliers.

With respect to commercial production of our potential products in the future, we plan on outsourcing production of the active pharmaceutical ingredients and final drug product manufacturing if they are approved for marketing by the applicable regulatory authorities. We have entered into a contractual relationship for the commercial final drug product manufacturing. However, we do not have any current contractual relationships for the commercial production of the active pharmaceutical ingredients.

We expect to continue to develop drug candidates that can be produced cost-effectively at contract manufacturing facilities. However, should a supplier or manufacturer on which we have relied to produce a product candidate provide us with a faulty product or such product is later recalled, we would likely experience delays and additional costs, each of which could be significant.

Intellectual Property

We have obtained patent protection for our primary product candidates, Rhopressa™ and Roclatan™ (patent protection for Roclatan™ arises from the patent protection we have secured for Rhopressa™), in the United States and certain foreign jurisdictions and are seeking patent protection in a number of other foreign jurisdictions for these product candidates. We intend to maintain and defend our patent rights to protect our technology, inventions, processes and improvements that are commercially important to the development of our business. We cannot be sure that any of our existing patents or patents we obtain in the future will be commercially useful in protecting our technology. We cannot be sure that our patents will issue on any of our pending patent applications or patent applications we file in the future. Our commercial success also depends in part on our non-infringement of the patents or proprietary rights of third parties. For a more comprehensive discussion of the risks related to our intellectual property, see “Risk Factors-Risks Related to Intellectual Property.”

Our intellectual property consists of issued patents, and pending patent applications for compositions of matter and methods of use, for our product candidates and other proprietary technology. For our primary product candidates Rhopressa™ and Roclatan™, we hold U.S. Patent 8,450,344, which is scheduled to expire in 2026, and U.S. Patent 8,394,826, which is scheduled to expire in 2030, each of which has claims directed to composition of matter and method of use. We hold patents for composition of matter and method of use in certain foreign jurisdictions for our primary product candidates. Additionally, we hold patents for other ROCK Inhibitor molecules.

We have established and continue to build proprietary positions for our product candidates and related technology in the United States and other jurisdictions. As of December 31, 2014, we had 44 United States or foreign issued patents that cover various aspects of our current and previously discontinued product candidates and our other proprietary technology and 22 U.S. patent applications or foreign patent applications that, if patents were to issue based on the existing claims, would cover various aspects of our current and previously discontinued product candidates and our other proprietary technology.

Aerie® is a registered trademark of ours and we have applications pending from the U.S. Patent and Trademark Office, or USPTO, for the registration of our trademarks Rhopressa™ and Roclatan™.

In October 2012, our board of directors authorized the divestiture of certain non-core intellectual property relating to implantable ophthalmic devices for future development by Novaer Holding, Inc., or Novaer, an independent company. In addition, as part of this transaction, we also licensed the non-ophthalmic rights to our intellectual property portfolio to Novaer. See Note 15 to our audited financial statements appearing elsewhere in this report.

Table of Contents

On September 6, 2013, we terminated our agreement to exclusively license to Novaer our intellectual property for non-ophthalmic indications. No consideration, or future obligation thereof, was exchanged in connection with this termination. Since September 6, 2013, we own all of the worldwide rights to our current product candidates for all indications, both ophthalmic and non-ophthalmic.

Regulatory Matters

FDA Regulation and Marketing Approval

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and related regulations. Drugs are also subject to other federal, state and local statutes and regulations. Failure to comply with the applicable United States regulatory requirements at any time during the product development process, approval process or after approval may subject an applicant to administrative or judicial sanctions and non-approval of product candidates. These sanctions could include the imposition by the FDA or an Institutional Review Board, or IRB, of a clinical hold on trials, the FDA's refusal to approve pending applications or related supplements, withdrawal of an approval, untitled or warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, restitution, disgorgement, civil penalties or criminal prosecution. Such actions by government agencies could also require us to expend a large amount of resources to respond to the actions. Any agency or judicial enforcement action could have a material adverse effect on us.

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products. These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, post-approval monitoring, advertising, promotion, sampling and import and export of our products. Our drugs must be approved by the FDA through the NDA process before they may be legally marketed in the United States. See “—The NDA Approval Process” below.

The process required by the FDA before drugs may be marketed in the United States generally involves the following:

- completion of non-clinical laboratory tests, animal studies and formulation studies conducted according to Good Laboratory Practices or other applicable regulations;
- submission of an IND, which allows clinical trials to begin unless FDA objects within 30 days;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug for its intended use or uses conducted in accordance with FDA regulations, Good Clinical Practices, or GCP, which are international ethical and scientific quality standards meant to assure the rights, safety and well-being of trial participants are protected and to define the roles of clinical trial sponsors, administrators, and monitors;
- pre-approval inspection of manufacturing facilities and clinical trial sites; and
- FDA approval of an NDA, which must occur before a drug can be marketed or sold.

IND and Clinical Trials

Prior to commencing the first clinical trial, an initial IND, which contains the results of preclinical tests along with other information, such as information about product chemistry, manufacturing and controls and a proposed protocol, must be submitted to the FDA. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA within the 30-day time period raises concerns or questions about the conduct of the clinical trial. In such a case, the IND sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin. A separate submission to the existing IND must be made for each successive clinical trial to be conducted during product development. Further, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial before it commences at that site. Informed written consent must also be obtained from each trial subject. Regulatory authorities, including the FDA, an IRB, a data safety monitoring board or the sponsor, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk or that the clinical trial is not being conducted in accordance with FDA requirements.

Table of Contents

For purposes of NDA approval, human clinical trials are typically conducted in sequential phases that may overlap:

Phase 1—the drug is initially given to healthy human subjects or patients and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. These trials may also provide early evidence on effectiveness. During Phase 1 clinical trials, sufficient information about the investigational drug’s pharmacokinetics and pharmacologic effects may be obtained to permit the design of well- controlled and scientifically valid Phase 2 clinical trials.

Phase 2—trials are conducted in a limited number of patients in the target population to identify possible adverse effects and safety risks, to determine the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more expensive Phase 3 clinical trials. Throughout this report, we refer to our initial Phase 2 clinical trials as “Phase 2a clinical trials” and our subsequent Phase 2 clinical trials as “Phase 2b clinical trials.”

Phase 3—when Phase 2 evaluations demonstrate that a dosage range of the product appears effective and has an acceptable safety profile, and provide sufficient information for the design of Phase 3 registration trials, Phase 3 registration trials are undertaken to provide statistically significant evidence of clinical efficacy and to further test for safety in an expanded patient population at multiple clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to further evaluate dosage, effectiveness and safety, to establish the overall benefit-risk relationship of the investigational drug and to provide an adequate basis for product labeling and approval by the FDA. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug.

All clinical trials must be conducted in accordance with FDA regulations, GCP requirements and their protocols in order for the data to be considered reliable for regulatory purposes.

An investigational drug product that is a combination of two different drugs in the same dosage form must comply with an additional rule that requires that each component make a contribution to the claimed effects of the drug product. This typically requires larger studies that test the drug against each of its components. In addition, typically, if a drug product is intended to treat a chronic disease, as is the case with our products, safety and efficacy data must be gathered over an extended period of time, which can range from six months to three years or more. Government regulation may delay or prevent marketing of product candidates or new drugs for a considerable period of time and impose costly procedures upon our activities.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

The NDA Approval Process

In order to obtain approval to market a drug in the United States, a marketing application must be submitted to the FDA that provides data establishing to the FDA’s satisfaction the safety and effectiveness of the investigational drug for the proposed indication. Each NDA submission requires a substantial user fee payment (currently exceeding \$2,300,000 for fiscal year 2015) unless a waiver or exemption applies. The application includes all relevant data available from pertinent non-clinical, preclinical and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product’s chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators that meet GCP requirements.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at

other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors

Table of Contents

typically use the end of Phase 2 meetings to discuss their Phase 2 clinical results and present their plans for the pivotal Phase 3 registration trial that they believe will support approval of the new drug.

Concurrent with clinical trials, companies usually complete additional animal safety studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in accordance with current Good Manufacturing Practice, or cGMP, requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drugs. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf-life.

The results of product development, non-clinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The FDA reviews all NDAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may request additional information rather than accept a NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. The FDA has 60 days from its receipt of an NDA to conduct an initial review to determine whether the application will be accepted for filing based on the agency's threshold determination that the application is sufficiently complete to permit substantive review. If the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA has agreed to specific performance goals on the review of NDAs and seeks to review standard NDAs in 12 months from submission of the NDA. The review process may be extended by the FDA for three additional months to consider certain late submitted information or information intended to clarify information already provided in the submission. After the FDA completes its initial review of an NDA, it will communicate to the sponsor that the drug will either be approved, or it will issue a complete response letter to communicate that the NDA will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, non-clinical or manufacturing data that must be received before the application can be approved, with no implication regarding the ultimate approvability of the application or the timing of any such approval, if ever. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. FDA has committed to reviewing such resubmissions in two to six months depending on the type of information included. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with GCP. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and often will request additional testing or information. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GCP, the FDA may determine the data generated by the clinical site should be excluded from the primary efficacy analyses provided in the NDA. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase 4 trials may be made a condition to be satisfied for continuing drug approval. The results of Phase 4 trials can confirm the effectiveness of a product candidate and can provide important safety information. In addition, the FDA now has express statutory authority to require sponsors to conduct post-marketing trials to specifically address safety issues identified by the agency. See “—Post-Marketing Requirements” below.

The FDA also has authority to require a Risk Evaluation and Mitigation Strategy, or a REMS, from manufacturers to ensure that the benefits of a drug outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the NDA submission. The need for a REMS is determined as part of the review of the NDA. Based on statutory standards, elements of a REMS may include “dear doctor letters,” a medication guide, more elaborate targeted educational programs, and in some cases elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. These elements

Table of Contents

are negotiated as part of the NDA approval, and in some cases if consensus is not obtained until after the PDUFA review cycle, the approval date may be delayed. Once adopted, REMS are subject to periodic assessment and modification.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution, or post-marketing trial requirements. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain, regulatory approval for our products, or obtaining approval but for significantly limited use, would harm our business. In addition, we cannot predict what adverse governmental regulations may arise from future U.S. or foreign governmental action.

The Hatch-Waxman Amendments

Under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, a portion of a product's U.S. patent term that was lost during clinical development and regulatory review by the FDA may be restored. The Hatch-Waxman Amendments also provide a process for listing patents pertaining to approved products in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (commonly known as the Orange Book) and for a competitor seeking approval of an application that references a product with listed patents to make certifications pertaining to such patents. In addition, the Hatch-Waxman Amendments provide for a statutory protection, known as non-patent exclusivity, against the FDA's acceptance or approval of certain competitor applications.

Patent Term Restoration

Patent term restoration can compensate for time lost during product development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application, provided the sponsor acted with diligence. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended and the extension must be applied for prior to expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug are then published in the FDA's Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval

is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a Section VIII statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

Table of Contents

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

An applicant submitting an NDA under Section 505(b)(2) of the FDCA, which permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference, is required to certify to the FDA regarding any patents listed in the Orange Book for the approved product it references to the same extent that an ANDA applicant would.

Market Exclusivity

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a Paragraph IV certification. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the non-clinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Post-Marketing Requirements

Following approval of a new product, a pharmaceutical company and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and recordkeeping activities, reporting to the applicable regulatory authorities of adverse experiences with the product, providing the regulatory authorities with updated safety and efficacy information, product sampling and distribution requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the drug's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities and requirements for promotional activities involving the internet. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses. Modifications or enhancements to the product or its labeling or changes of the site of manufacture are often subject to the approval of the FDA and other regulators, who may or may not grant approval or may include in a lengthy review process.

Prescription drug advertising is subject to federal, state and foreign regulations. In the United States, the FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act, or the PDMA, a part of the FDCA.

In the United States, once a product is approved, its manufacture is subject to comprehensive and continuing regulation by the FDA. The FDA regulations require that products be manufactured in specific approved facilities and

in accordance with cGMP. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and

Table of Contents

quality assurance activities. NDA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms, and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by the FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such product or may result in restrictions on a product, manufacturer, or holder of an approved NDA, including, among other things, recall or withdrawal of the product from the market.

In addition, the manufacturer and/or sponsor under an approved NDA are subject to annual product and establishment fees, currently exceeding \$110,000 per product and \$569,000 per establishment for fiscal year 2015. These fees are typically increased annually.

The FDA also may require post-marketing testing, also known as Phase 4 testing, REMS to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from the FDA, mandated corrective advertising or communications with doctors, withdrawal of approval, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

Reimbursement, Anti-Kickback and False Claims Laws and Other Regulatory Matters

In the United States, the research, manufacturing, distribution, sale and promotion of drug products and medical devices are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare & Medicaid Services, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency, state Attorneys General and other state and local government agencies. For example, sales, marketing and scientific/educational grant programs must comply with the Federal Anti-Kickback Statute, the False Claims Act, as amended, the privacy regulations promulgated under the Health Insurance Portability and Accountability Act (HIPAA), as amended, and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. The handling of any controlled substances must comply with the U.S. Controlled Substances Act and Controlled Substances Import and Export Act. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Unlike Medicare Part A and B, part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive regulatory approval. However, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we

might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-government payors.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. A plan for the research will be developed by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures will be made to Congress. Although the results of the comparative

Table of Contents

effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidate, if any such product or the condition that it is intended to treat is the subject of a trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our product candidate. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally tend to be significantly lower.

As noted above, in the United States, we are subject to complex laws and regulations pertaining to healthcare "fraud and abuse," including, but not limited to, the Federal Anti-Kickback Statute, the Federal False Claims Act, and other state and federal laws and regulations. The Federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties, and exclusion from participation in federal healthcare programs. In addition, many states have adopted laws similar to the Federal Anti-Kickback Statute. Some of these state prohibitions apply to the referral of patients for healthcare services reimbursed by any insurer, not just federal healthcare programs such as Medicare and Medicaid. Due to the breadth of these federal and state anti-kickback laws, the absence of guidance in the form of regulations or court decisions, and the potential for additional legal or regulatory change in this area, it is possible that our future sales and marketing practices and/or our future relationships with eye-care professionals might be challenged under anti-kickback laws, which could harm us. Because we intend to commercialize products that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject.

The Federal False Claims Act prohibits anyone from knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. Although we would not submit claims directly to payors, manufacturers can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our products, are subject to scrutiny under this law. For example, pharmaceutical companies have been found liable under the Federal False Claims Act in connection with their off-label promotion of drugs. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties of between \$5,500 and \$11,000 for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and, although the Federal False Claims Act is a civil statute, conduct that results in a False Claims Act violation may also implicate various federal criminal statutes. If the government were to allege that we were, or convict us of, violating these false claims laws, we could be subject to a

substantial fine and may suffer a decline in our stock price. In addition, private individuals have the ability to bring actions under the Federal False Claims Act and certain states have enacted laws modeled after the Federal False Claims Act.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. In addition, as discussed below, beginning in 2013, a similar federal requirement will require manufacturers to track and report to the federal government certain payments made to physicians and teaching hospitals made in the previous calendar year. These laws may affect our sales, marketing and other promotional activities by imposing administrative and compliance burdens on us. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state, and soon federal, authorities.

Table of Contents

The failure to comply with regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by required, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Patient Protection and Affordable Care Act

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, PPACA) was enacted, which includes measures that have or will significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of PPACA of greatest importance to the pharmaceutical industry are the following:

The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services a condition for states to receive federal matching funds for the manufacturer's covered outpatient drugs furnished to Medicaid patients. Effective in 2010, PPACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs and biologic agents to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. PPACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization as of 2010 and by, beginning in 2011, expanding the population potentially eligible for Medicaid drug benefits. The Centers for Medicare & Medicaid Services, or CMS, have proposed to expand Medicaid rebate liability to the territories of the United States as well. In addition, PPACA provides for the public availability of retail survey prices and certain weighted average AMPs under the Medicaid program. The implementation of this requirement by the CMS may also provide for the public availability of pharmacy acquisition of cost data, which could negatively impact our sales.

In order for a pharmaceutical product to receive federal reimbursement under the Medicare Part B and Medicaid programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. Effective in 2010, PPACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs when used for the orphan indication. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

Effective in 2011, PPACA imposed a requirement on manufacturers of branded drugs and biologic agents to provide a 50% discount off the negotiated price of branded drugs dispensed to Medicare Part D patients in the coverage gap (i.e., "donut hole").

Effective in 2011, PPACA imposed an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications.

PPACA requires pharmaceutical manufacturers to track certain financial arrangements with physicians and teaching hospitals, including any "transfer of value" made or distributed to such entities, as well as any investment interests held by physicians and their immediate family members. Manufacturers were required to track this information beginning

in 2013, and the first reports were due in 2014. The information reported each year is made publicly available on a searchable website.

Table of Contents

As of 2010, a new Patient-Centered Outcomes Research Institute was established pursuant to PPACA to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products.

PPACA created the Independent Payment Advisory Board, which has the authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. Under certain circumstances, these recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings.

PPACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation from 2011 to 2019.

Many of the details regarding the implementation and impact of PPACA are yet to be determined, and at this time, it remains unclear the full effect that PPACA would have on our business.

European Union Drug Development

In the European Union, our products will also be subject to extensive regulatory requirements. As in the United States, medicinal products can only be marketed if a marketing authorization from the competent regulatory agencies has been obtained, and the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the EU clinical trial regulatory framework, setting out common rules for the control and authorization of clinical trials in the European Union, the EU Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved in each of the EU countries where the trial is to be conducted by two distinct bodies: the National Competent Authority, or NCA, and one or more Ethics Committees, or ECs. In addition, all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the NCA and ECs of the Member State where they occurred.

The EU clinical trials legislation is currently undergoing a revision process mainly aimed at making more uniform and streamlining the clinical trials authorization process, simplifying adverse event reporting procedures, improving the supervision of clinical trials and increasing the transparency of clinical trials.

European Union Drug Review Approval

In the European Economic Area, or EEA, which is comprised of the 27 Member States of the European Union plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations: the Community MA, which is issued by the European Commission through the Centralized Procedure based on the opinion of the Committee for Medicinal Products for Human Use, or CHMP, a body of the EMA, and which is valid throughout the entire territory of the EEA; and the National MA, which is issued by the competent authorities of the Member States of the EEA and only authorized marketing in that Member State's national territory and not the EEA as a whole.

The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products and medicinal products containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the European Union. The National MA is for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent

authorities of each of the Member States in which the MA is sought, one of which is selected by the applicant as the Reference Member state, or RMS. If the RMS proposes to authorize the product, and the other Member States do not raise objections, the product is granted a national MA in all the Member States where the authorization was sought. Before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

Table of Contents

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

Employees

We had 40 full-time employees as of December 31, 2014. None of our employees are represented by any collective bargaining unit. We believe that we maintain good relations with our employees.

Corporate and Available Information

Our principal executive offices are located at 2030 Main Street, Suite 1500, Irvine, California 92614 and our telephone number is (949) 526-8700. We were incorporated in Delaware in June 2005. Our internet address is www.aeriepharma.com. We make available on our website, free of charge, our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Our SEC reports can be accessed through the Investors section of our website. Further, a copy of this Annual Report on Form 10-K is located at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D. C. 20549. Information on the operation of the Public Reference Room can be obtained by calling the SEC at 1-800-SEC-0330. The SEC maintains a website that contains reports, proxy and information statements and other information regarding our filings at www.sec.gov. The information found on our website is not incorporated by reference into this report or any other report we file with or furnish to the SEC.

Table of Contents

ITEM 1A. RISK FACTORS

We operate in an industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the trading price of our common stock to decline.

Risks Related to Development, Regulatory Approval and Commercialization

We depend substantially on the success of our product candidates, particularly Rhopressa™ and Roclatan™, which are still in development. If we are unable to successfully commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

Our business and the ability to generate revenue related to product sales, if ever, will depend on the successful development, regulatory approval and commercialization of our product candidates for the treatment of patients with glaucoma and other diseases of the eye, particularly Rhopressa™ and Roclatan™, which are still in development, and other potential products we may develop or license. We have invested a significant portion of our efforts and financial resources in the development of our existing product candidates. The success of our product candidates will depend on several factors, including:

- successful completion of clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity;
- protecting our rights in our intellectual property;
- launching commercial sales of our product candidates, if and when approved;
 - obtaining reimbursement from third-party payors for product candidates, if and when approved;
- competition with other products; and
- continued acceptable safety profile for our product candidates following regulatory approval, if and when received.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business and we may not be able to earn sufficient revenues and cash flows to continue our operations.

We have not obtained regulatory approval for any of our product candidates in the United States or any other country. We currently do not have any product candidates that have gained regulatory approval for sale in the United States or any other country, and we cannot guarantee that we will ever have marketable products. Our business is substantially dependent on our ability to complete the development of, obtain regulatory approval for and successfully commercialize product candidates in a timely manner. We cannot commercialize product candidates in the United States without first obtaining regulatory approval to market each product from the FDA; similarly, we cannot commercialize product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Phase 3 trials for Rhopressa™ commenced in July 2014 and Roclatan™ is planned to be advanced into Phase 3 clinical trials in mid-2015. We cannot predict whether these trials and future trials will be successful or whether regulators will agree with our conclusions regarding the preclinical studies and clinical trials we have conducted to date.

Before obtaining regulatory approvals for the commercial sale of any product candidate for a target indication, we must demonstrate in preclinical studies and well-controlled clinical trials, and, with respect to approval in the United States, to the satisfaction of the FDA, that the product candidate is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. In the United States, we have not submitted an NDA for any of our product candidates. An NDA must include extensive preclinical and clinical data and

supporting information to establish the product candidate's safety and effectiveness for each desired indication. The NDA must also include significant information regarding the chemistry, manufacturing and controls for the product. Obtaining approval of an NDA is a lengthy, expensive and uncertain process, and approval may not be obtained. If we submit an NDA to the FDA, the FDA must decide whether to accept

Table of Contents

or reject the submission for filing. We cannot be certain that any submissions will be accepted for filing and review by the FDA.

Regulatory authorities outside of the United States, such as in Europe and Japan and in emerging markets, also have requirements for approval of drugs for commercial sale with which we must comply prior to marketing in those areas. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and obtaining regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could require additional non-clinical studies or clinical trials, which could be costly and time consuming. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. For all of these reasons, we may not obtain foreign regulatory approvals on a timely basis, if at all.

The process to develop, obtain regulatory approval for and commercialize product candidates is long, complex and costly both inside and outside of the United States, and approval is never guaranteed. Even if our product candidates were to successfully obtain approval from the regulatory authorities, any approval might significantly limit the approved indications for use, or require that precautions, contraindications, or warnings be included on the product labeling, or require expensive and time-consuming post-approval clinical studies or surveillance as conditions of approval. Following any approval for commercial sale of our product candidates, certain changes to the product, such as changes in manufacturing processes and additional labeling claims, will be subject to additional FDA review and approval. Also, regulatory approval for any of our product candidates may be withdrawn. If we are unable to obtain regulatory approval for our product candidates in one or more jurisdictions, or any approval contains significant limitations, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed. Furthermore, we may not be able to obtain sufficient funding or generate sufficient revenue and cash flows to continue the development of any other product candidate in the future.

Regulatory approval may be substantially delayed or may not be obtained for one or all of our product candidates if regulatory authorities require additional time or studies to assess the safety and efficacy of our product candidates. We may be unable to initiate or complete development of our product candidates on schedule, if at all. If regulatory authorities require additional time or studies to assess the safety or efficacy of our product candidates, we may require funding beyond the amounts currently on our balance sheet. In addition, in the event of any unforeseen costs or other business decisions, we may not have or be able to obtain adequate funding to complete the necessary steps for approval for any or all of our product candidates. Preclinical studies and clinical trials required to demonstrate the safety and efficacy of our product candidates are time consuming and expensive and together take several years or more to complete. Delays in regulatory approvals or rejections of applications for regulatory approval in the United States, Europe, Japan or other markets may result from many factors, including:

- our inability to obtain sufficient funds required for a clinical trial;
- regulatory requests for additional analyses, reports, data, non-clinical and preclinical studies and clinical trials;
- regulatory questions regarding interpretations of data and results and the emergence of new information regarding our product candidates or other products;
- clinical holds, other regulatory objections to commencing or continuing a clinical trial or the inability to obtain regulatory approval to commence a clinical trial in countries that require such approvals;
- failure to reach agreement with the FDA or non-U.S. regulators regarding the scope or design of our clinical trials;
- our inability to enroll a sufficient number of patients who meet the inclusion and exclusion criteria in our clinical trials;
- our inability to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- unfavorable or inconclusive results of clinical trials and supportive non-clinical studies, including unfavorable results regarding effectiveness of product candidates during clinical trials;
- any determination that a clinical trial presents unacceptable health risks;

lack of adequate funding to continue the clinical trial due to unforeseen costs or other business decisions;

31

Table of Contents

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

- our inability to identify and maintain a sufficient number of sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indications targeted by our product candidates;
- our inability to obtain approval from institutional review boards to conduct clinical trials at their respective sites;
- our inability to timely manufacture or obtain from third parties sufficient quantities or quality of the product candidate or other materials required for a clinical trial; and
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data.

Changes in regulatory requirements and guidance may also occur and we may need to amend clinical trial protocols submitted to applicable regulatory authorities to reflect these changes. Amendments may require us to resubmit clinical trial protocols to institutional review boards for re-examination, which may impact the costs, timing or successful completion of a clinical trial.

If we are required to conduct additional clinical trials or other studies with respect to any of our product candidates beyond those that we initially contemplated, if we are unable to successfully complete our clinical trials or other studies or if the results of these studies are not positive or are only modestly positive, we may be delayed in obtaining regulatory approval for that product candidate, we may not be able to obtain regulatory approval at all or we may obtain approval for indications that are not as broad as intended. Our product development costs will also increase if we experience delays in testing or approvals and we may not have sufficient funding to complete the testing and approval process. Significant clinical trial delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products if and when approved. If any of this occurs, our business will be materially harmed.

If we are unable to establish a direct sales force in North America and possibly Europe, our business may be harmed. We currently do not have an established sales organization and do not have a marketing or distribution infrastructure. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. If our product candidates are approved by the FDA for commercial sale, we intend to market directly to eye-care professionals in North America through our own sales force, targeting approximately 10,000 high-prescribing eye-care professionals. If our product candidates are approved in Europe for commercial sale and if we self-commercialize our product candidates in Europe, we will need to establish similar functions or outsource these functions to third parties. We will need to incur significant additional expenses and commit significant additional time and management resources to establish and train a sales force to market and sell our products. We may not be able to successfully establish these capabilities despite these additional expenditures. Factors that may inhibit our efforts to successfully establish a sales force include:

- our inability to compete with other pharmaceutical companies to recruit, hire, train and retain adequate numbers of effective sales and marketing personnel with requisite knowledge of our target market;
- the inability of sales personnel to obtain access to adequate numbers of eye-care professionals to prescribe any future approved products;
- unforeseen costs and expenses associated with creating an independent sales and marketing organization; and
- a delay in bringing products to market after efforts to hire and train our sales force have already commenced.

In the event we are unable to successfully market and promote our products, our business may be harmed.

We currently intend to explore the licensing of commercialization rights or other forms of collaboration outside of North America and possibly Europe, which will expose us to additional risks of conducting business in international markets.

Markets outside of North America are an important component of our growth strategy. If we fail to commercialize, obtain licenses or enter into collaboration arrangements with selling parties, or if these parties are not successful, our revenue-generating growth potential will be adversely affected. Moreover, international business relationships subject us to additional risks that may materially adversely affect our ability to attain or sustain profitable operations,

including:

32

Table of Contents

efforts to enter into collaboration or licensing arrangements with third parties in connection with our international sales, marketing and distribution efforts may increase our expenses or divert our management's attention from the acquisition or development of product candidates;

- changes in a specific country's or region's political and cultural climate or economic condition;
- differing regulatory requirements for drug approvals and marketing internationally;
- difficulty of effective enforcement of contractual provisions in local jurisdictions;
- potentially reduced protection for intellectual property rights;
- potential third-party patent rights in countries outside of the United States;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability, particularly in non-U.S. economies and markets, including several countries in Europe;
- compliance with tax, employment, immigration and labor laws for employees traveling abroad;
- the effects of applicable foreign tax structures and potentially adverse tax consequences;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incidental to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- failure of our employees and contracted third parties to comply with Office of Foreign Asset Control rules and regulations and the Foreign Corrupt Practices Act;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;

and

- business interruptions resulting from geo-political actions, including war and terrorism, or natural disasters, including earthquakes, volcanoes, typhoons, floods, hurricanes and fires.

These and other risks may materially adversely affect our ability to attain or sustain revenue from international markets.

Failure can occur at any stage of clinical development. If the clinical trials for our current and potential future product candidates are unsuccessful, we could be required to abandon development.

A failure of one or more clinical trials can occur at any stage of testing for a variety of reasons. The outcome of preclinical testing and early clinical trials may not be predictive of the outcome of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. In addition, adverse events may occur or other risks may be discovered in Phase 3 clinical trials that may cause us to suspend or terminate our clinical trials. In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same product candidate due to numerous factors, including changes in or adherence to trial protocols, differences in size and type of the patient populations and the rates of dropout among clinical trial participants. Our future clinical trial results therefore may not demonstrate safety and efficacy sufficient to obtain regulatory approval for our current and potential future product candidates.

Flaws in the design of a clinical trial may not become apparent until the clinical trial is well-advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support regulatory approval. In addition, clinical trials often reveal that it is not practical or feasible to continue development efforts. Further, we have never submitted an NDA for any potential products.

We may voluntarily suspend or terminate our clinical trials if at any time we believe that they present an unacceptable risk to participants. Further, regulatory agencies, institutional review boards or data safety monitoring boards may at any time order the temporary or permanent discontinuation of our clinical trials or request that we cease using investigators in the clinical trials if they believe that the clinical trials are not being conducted in accordance with applicable regulatory requirements, or that they present an unacceptable safety risk to participants. Since our inception, we have not voluntarily or involuntarily suspended or terminated a clinical trial due to unacceptable safety risks to participants.

Table of Contents

If the results of our clinical trials for our current product candidates or clinical trials for any future product candidates do not achieve the primary efficacy endpoints or demonstrate unexpected safety issues, the prospects for approval of our product candidates will be materially adversely affected. Moreover, preclinical and clinical data are often susceptible to varying interpretations, analyses and entry criteria, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have failed to achieve similar results in later clinical trials, including longer term trials, or have failed to obtain regulatory approval of their product candidates. While our Phase 2b clinical trial demonstrated statistical non-inferiority of Rhopressa™ to the leading PGA, latanoprost, in the sub-group with moderately elevated IOP, our Phase 3 registration trials for Rhopressa™ do not include a PGA as a comparator. Furthermore, the entry criteria for our Phase 3 registration trials for Rhopressa™ were limited to patients with IOPs greater than 20 mmHg and less than 27 mmHg, and our Phase 2b clinical trial for Rhopressa™ did not include patients with IOPs outside of the range from 22 mmHg to 36 mmHg.

Many compounds that initially showed promise in clinical trials or earlier stage testing have later been found to cause undesirable or unexpected adverse effects that have prevented further development of the compound. Our clinical trials for our primary product candidates, Rhopressa™ and Roclatan™, may not produce the results that we expect. In addition, if based on clinical results of Rhopressa™, we discontinue the advancement of this product candidate, in certain circumstances we may similarly determine not to advance Roclatan™, which combines Rhopressa™ with latanoprost. Our clinical trials are also designed to test the use of Rhopressa™ and Roclatan™ as a monotherapy, rather than as an add-on therapy. Accordingly, the efficacy of our primary product candidates may not be similar or correspond directly to their efficacy when used as an add-on therapy.

Several companies have previously pursued ROCK inhibitors for ophthalmic use but to date no ROCK inhibitors have been approved and most of those companies have chosen to discontinue clinical development of their ROCK inhibitors. One of our ROCK inhibitors, AR-12286, was discontinued in the clinical stage of development due to an inability to maintain its effectiveness over time. In a 28-day Phase 2b clinical trial, AR-12286 lowered IOP by 6.7 mmHg on day seven, but lowered IOP by only 5.3 mmHg on day 28. This trend continued in a follow-up three-month study. As a result, in June 2013 we discontinued any further clinical development of AR-12286 and its fixed-dose combination product PG286.

Phase 2b trials for Rhopressa™ and Roclatan™ did not show a loss of efficacy over time. Similarly, published clinical data for other ROCK inhibitors have not shown a loss of efficacy over time. However, we have not previously conducted a three-month Phase 2b clinical trial for Rhopressa™ or Roclatan™, and therefore there can be no assurance as to the efficacy of Rhopressa™ or Roclatan™ beyond 28 days. In addition, our current product candidates remain subject to the risks associated with clinical drug development as indicated above.

In February 2014, we reported the results of a preclinical animal study sponsored by Aerie, whereby the administration of Rhopressa™ eye drops demonstrated statistically significant reductions in EVP and IOP in rabbits following the third daily dose. Based on the results of this preclinical study, together with the consistent IOP-lowering effect of Rhopressa™ demonstrated in our clinical trials to date, we believe the reduction of EVP is an additional MOA of Rhopressa™ and Roclatan™. However, like the other differentiated MOAs of our product candidates, increasing outflow through the TM and decreasing fluid production in the eye, our product candidates' effect on EVP has not been studied in humans and neither our ongoing Phase 3 registration trials for Rhopressa™ nor our planned Phase 3 clinical trials for Roclatan™ will be designed to demonstrate reduction of EVP or other MOAs of our product candidates. If we are not able to demonstrate to the satisfaction of the FDA and relevant non-U.S. regulators the reduction of EVP, or any of the other differentiated MOAs of our product candidates, even if we otherwise obtain regulatory approval for Rhopressa™ and Roclatan™, it could limit the types of claims we will be able to make in our marketing and labeling of our product candidates.

Additionally, we believe Rhopressa™, if approved, will compete against PGAs in several populations including where patients have low to moderately elevated IOPs at the time of diagnosis. No patients with low-tension glaucoma have been or will be included in these clinical studies, and our expectations with respect to subjects with low IOP are based to a large extent on extrapolation of results for subjects with moderately elevated IOP. Even if our product candidates were to obtain regulatory approval, if we are unable to support claims about our product candidates to the

satisfaction of the FDA and relevant non-U.S. regulators, including claims with respect to the efficacy of our product candidates for patients with low IOP, it could limit the types of claims we will be able to make in our marketing and product labeling of these product candidates.

In addition to the circumstances noted above, we may experience numerous unforeseen events that could cause our clinical trials to be delayed, suspended or terminated, or which could delay or prevent our ability to receive regulatory approval or commercialize our current and potential future product candidates, including:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or implement a clinical hold;

Table of Contents

- the number of patients required for clinical trials of our product candidates may be larger than we anticipate,
- enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may elect or be required to suspend or terminate clinical trials of our product candidates based on a finding that the participants are being exposed to health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable adverse effects or other unexpected characteristics.

If we elect or are required to suspend or terminate a clinical trial of any of our current and potential future product candidates, our commercial prospects will be adversely impacted and our ability to generate product revenues may be delayed or eliminated.

Our product candidates may have undesirable adverse effects, which may delay or prevent regulatory approval or, if approval is received, require our products to be taken off the market, require them to include safety warnings or otherwise limit their sales.

Unforeseen adverse effects from any of our product candidates could arise either during clinical development or, if approved, after the approved product has been marketed. To date, the main tolerability finding of Rhopressa™ has been transient hyperemia, or eye-redness. Roclatan™ combines Rhopressa™ with latanoprost. To date, the main tolerability finding of Roclatan™ has been mild hyperemia, or eye redness. The main adverse effects of latanoprost include hyperemia, irreversible change in iris color, discoloration of the skin around the eyes and droopiness of eyelids caused by the loss of orbital fat.

Any undesirable adverse effects that may be caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale. In addition, if any of our product candidates receives regulatory approval and we or others later identify undesirable adverse effects caused by the product, we could face one or more of the following consequences:

- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication, or other labeling changes;
- regulatory authorities may withdraw their approval of the product;
- regulatory authorities may seize the product;
- we may be required to change the way that the product is administered, conduct additional clinical trials or recall the product;
- we may be subject to litigation or product liability claims fines, injunctions, or criminal penalties; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing such product, which in turn could delay or prevent us from generating significant revenues from its sale.

Table of Contents

We face competition from established branded and generic pharmaceutical companies and if our competitors are able to develop and market products that are preferred over our products, our commercial opportunity will be reduced or eliminated.

The development and commercialization of new drug products is highly competitive. We face competition from established branded and generic pharmaceutical companies, as well as from academic institutions, government agencies and private and public research institutions, which may in the future develop products to treat patients with glaucoma. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. In early 2013, Sucampo Pharmaceuticals, Inc. commercially relaunched Rescula, a twice-daily dosed PGA, with the claim that it reduces elevated IOP by increasing the outflow of aqueous humor through the TM. Additionally, Bausch + Lomb Inc., a wholly owned subsidiary of Valeant Pharmaceuticals International, Inc., is developing a nitric oxide-donating latanoprost and is currently in Phase 3 clinical trials. Early-stage companies are also developing glaucoma treatments and may prove to be significant competitors, such as Inotek Pharmaceuticals, which is developing an adenosine receptor agonist. We expect that our competitors will continue to develop new glaucoma treatments, which may include eye drops, oral treatments, surgical procedures, implantable devices or laser treatments. Alternative treatments beyond eye drops continue to develop. For example, although surgical procedures are currently used in severe cases, less invasive procedures are currently under development and we expect that we will compete with other companies that develop implantable devices or other products or procedures for use in the treatment of glaucoma.

Other early-stage companies may also compete through collaborative arrangements with large and established companies. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity will be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer adverse effects, are more convenient or are less expensive than our potential products. We expect that our ability to compete effectively will depend upon, among other things, our ability to:

- successfully complete clinical trials and obtain all requisite regulatory approvals in a timely and cost-effective manner;
- obtain and maintain patent protection and non-patent exclusivity for our products and otherwise prevent the introduction of generics of our products;
- attract and retain key personnel;
- build an effective selling and marketing infrastructure;
- demonstrate the advantages of our product candidates compared to alternative therapies, including currently marketed PGA and non-PGA products;
- compete against other products with fewer contraindications; and
- obtain and sustain adequate reimbursement from third-party payors.

If our competitors market products that are more effective, safer, have fewer side effects or are less expensive than our potential products or that reach the market sooner than our future products, if any, we may not achieve commercial success.

The commercial success of our potential products will depend on the degree of market acceptance among eye-care professionals, patients, patient advocacy groups, healthcare payors and the medical community.

Our potential products may not gain market acceptance among eye-care professionals, patients, patient advocacy groups, healthcare payors and the medical community. There are a number of available therapies marketed for the treatment of glaucoma. Some of these drugs are branded and subject to patent protection, but most others, including latanoprost and many beta blockers, are available on a generic basis. Many of these approved drugs are well established therapies and are widely accepted by eye-care professionals, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. The degree of market acceptance of our potential products will depend on a number of factors, including:

- the market price, affordability and patient out-of-pocket costs of our potential products relative to other available products, which are predominantly generics;
- the effectiveness of our potential products as compared with currently available products;
- patient willingness to adopt our potential products in place of current therapies;

36

Table of Contents

- varying patient characteristics including demographic factors such as age, health, race and economic status;
- changes in the standard of care for the targeted indications for any of our product candidates;
- the prevalence and severity of any adverse effects;
- limitations or warnings contained in a product candidate's FDA-approved labeling;
- limitations in the approved clinical indications and MOAs for our product candidates;
- relative convenience and ease of administration;
- the strength of our selling, marketing and distribution capabilities;
- the quality of our relationship with patient advocacy groups;
- sufficient third-party coverage or reimbursement; and
- potential product liability claims.

In addition, the potential market opportunity for our potential products is difficult to precisely estimate. Our estimates of the potential market opportunity for our potential products include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, independent sources have not verified all of our assumptions. If any of these assumptions proves to be inaccurate, then the actual market for our potential products could be smaller than our estimates of our potential market opportunity. If the actual market for our potential products is smaller than we expect, our product revenue may be limited, and it may be more difficult for us to achieve or maintain profitability. If we fail to achieve market acceptance of our potential products in the United States and abroad, our revenue will be more limited and it will be more difficult to achieve profitability.

If we fail to obtain and sustain an adequate level of reimbursement for our potential products by third-party payors, potential future sales would be materially adversely affected.

The course of treatment for glaucoma patients includes primarily older drugs, and the leading products for the treatment of glaucoma currently in the market, including latanoprost and timolol, are available as generic brands. There will be no commercially viable market for our potential products without reimbursement from third-party payors, and any reimbursement policy may be affected by future healthcare reform measures. We cannot be certain that reimbursement will be available for our potential products or any other product candidate we develop.

Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our anticipated revenue and gross margins will be adversely affected.

Third-party payors, such as government or private healthcare insurers, carefully review and increasingly question and challenge the coverage of and the prices charged for drugs. Reimbursement rates from private health insurance companies vary depending on the company, the insurance plan and other factors. Reimbursement rates may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. A current trend in the United States healthcare industry is toward cost containment. Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, may question the coverage of, and challenge the prices charged for, medical products and services, and many third-party payors limit coverage of or reimbursement for newly approved healthcare products. In particular, third-party payors may limit the covered indications. Cost-control initiatives could decrease the price we might establish for products, which could result in product revenues being lower than anticipated. We believe our drugs will be priced significantly higher than existing generic drugs and consistently with current branded drugs. If we are unable to show a significant benefit relative to existing generic drugs, Medicare, Medicaid and private payors may not be willing to reimburse for our drugs, which would significantly reduce the likelihood of them gaining market acceptance. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. We expect that private insurers will consider the efficacy, cost effectiveness, safety and tolerability of our potential products in determining whether to approve reimbursement for such products and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we

do not receive approval for reimbursement of our potential products from private insurers on a timely or satisfactory basis. Limitations on coverage could also be imposed at the local Medicare carrier level or by fiscal intermediaries. Medicare Part D, which provides a pharmacy benefit to Medicare patients as discussed below, does not require participating prescription drug plans to cover all drugs within

Table of Contents

a class of products. Our business could be materially adversely affected if Part D prescription drug plans were to limit access to, or deny or limit reimbursement of, our product candidates or other potential products.

Reimbursement in the European Union must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The negotiation process in some countries can exceed 12 months. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our products to other available therapies. If the prices for our potential products decrease or if governmental and other third-party payors do not provide adequate coverage and reimbursement levels, our revenue, potential for future cash flows and prospects for profitability will suffer. If we are found in violation of federal or state “fraud and abuse” laws or other healthcare laws and regulations, we may be required to pay a penalty and/or be suspended from participation in federal or state healthcare programs, which may adversely affect our business, financial condition and results of operation.

In the United States, we are subject to various federal and state healthcare “fraud and abuse” laws, including anti-kickback laws, false claims laws and other laws intended, among other things, to reduce fraud and abuse in federal and state healthcare programs. The Federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Although we seek to structure our business arrangements in compliance with all applicable requirements, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the Federal Anti-Kickback Statute. The Federal False Claims Act prohibits anyone from, among other things, knowingly presenting or causing to be presented for payment to the government, including the federal healthcare programs, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Many states have similar false claims laws. Cases have been brought under false claims laws alleging that off-label promotion of pharmaceutical products or the provision of kickbacks have resulted in the submission of false claims to governmental healthcare programs. Under the Health Insurance Portability and Accountability Act of 1996, we are prohibited from knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payors, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services to obtain money or property of any healthcare benefit program. Violations of fraud and abuse laws may be punishable by criminal and/or civil sanctions, including penalties, fines and/or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private individuals have the ability to bring actions on behalf of the government under the Federal False Claims Act as well as under the false claims laws of several states.

Many states have adopted laws similar to the Federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payors. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America’s Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties.

Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. While we believe we have structured our business arrangements to comply with these laws, it is possible that the government could

allege violations of, or convict us of violating, these laws. If we are found in violation of one of these laws, we could be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from governmental funded federal or state healthcare programs and the curtailment or restructuring of our operations. Were this to occur, our business, financial condition and results of operations and cash flows may be materially adversely affected.

Table of Contents

Recently enacted and future legislation may increase the difficulty and cost of commercializing our potential products and may affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our potential products, restrict or regulate post-marketing activities and affect our ability to profitably sell our potential products for which we obtain regulatory approval.

In the United States, the MMA changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly by establishing Medicare Part D and introduced a new reimbursement methodology based on average sales prices for physician-administered drugs under Medicare Part B. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class under the new Part D program. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and reimbursement rate that we receive for any of our approved products.

While the MMA only applies to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the PPACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. PPACA increased manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate amount for both branded and generic drugs and revised the definition of "average manufacturer price," or AMP, which may also increase the amount of Medicaid drug rebates manufacturers are required to pay to states. The legislation also expanded Medicaid drug rebates, which previously had been payable only on fee-for-service utilization, to Medicaid managed care utilization, and created an alternative rebate formula for certain new formulations of certain existing products that is intended to increase the rebates due on those drugs. The Centers for Medicare & Medicaid Services, which administers the Medicaid Drug Rebate Program, also has proposed to expand Medicaid rebates to the utilization that occurs in the territories of the United States, such as Puerto Rico and the Virgin Islands. Further, beginning in 2011, PPACA imposed a significant annual fee on companies that manufacture or import branded prescription drug products and requires manufacturers to provide a 50% discount off the negotiated price of prescriptions filled by beneficiaries in the Medicare Part D coverage gap, referred to as the "donut hole." Substantial new provisions affecting compliance have also been enacted, which may require us to modify our business practices with healthcare practitioners. For example, pharmaceutical companies are required to track certain payments made to physicians, and the first reports were due in 2014 and the reported information was made publicly available on a searchable website in September 2014. We will not know the full effects of PPACA until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the full effect of PPACA, the new law appears likely to continue the downward pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Legislative and regulatory proposals have been introduced at both the state and federal level to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing approval testing and other requirements.

If we face allegations of noncompliance with the law and encounter sanctions, our reputation, revenues and liquidity may suffer, and our products could be subject to restrictions or withdrawal from the market.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may

significantly and adversely affect our ability to commercialize and generate revenues from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected. Additionally, if we are unable to generate revenues from our product sales, our potential for achieving profitability will be diminished and the capital necessary to fund our operations will be increased.

Table of Contents

If our product candidates receive regulatory approval, we will be subject to ongoing regulatory requirements and we may face future development, manufacturing and regulatory difficulties.

Our product candidates, if approved, will also be subject to ongoing regulatory requirements for labeling, packaging, storage, advertising, promotion, sampling, record-keeping, submission of safety and other post-market approval information, importation and exportation. In addition, approved products, manufacturers and manufacturers' facilities are required to comply with extensive FDA and EMA, requirements and the requirements of other similar agencies, including ensuring that quality control and manufacturing procedures conform to cGMP requirements. As such, we and our potential future contract manufacturers will be subject to continual review and periodic inspections to assess compliance with cGMPs. Accordingly, we and others with whom we work will be required to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control. We will also be required to report certain adverse reactions and production problems, if any, to the FDA and EMA and other similar agencies and to comply with certain requirements concerning advertising and promotion for our potential products. Promotional communications with respect to prescription drugs also are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Accordingly, once approved, we may not promote our products, if any, for indications, uses or claims for which they are not approved.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, it may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If our potential products fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- require product recalls;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us or our potential future collaborators to enter into a consent decree or permanent injunction, which can include shutdown of manufacturing facilities, imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- impose other administrative or judicial civil or criminal penalties or pursue criminal prosecution;
- withdraw regulatory approval;
- refuse to approve pending applications or supplements to approved applications filed by us or by our potential future collaborators;
- impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products.

We may not be able to identify additional therapeutic opportunities for our potential product candidates or to expand our portfolio of products.

We may explore other therapeutic opportunities with ROCK inhibition in ophthalmology and seek to commercialize a portfolio of new ophthalmic drugs in addition to our product candidates that we are currently developing.

Research programs to pursue the development of our product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not we ultimately are successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective drugs; or

it may take greater human and financial resources to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs than we will possess, thereby limiting our ability to diversify and expand our product portfolio.

40

Table of Contents

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or any uses for our existing proprietary compounds beyond glaucoma or to develop suitable potential product candidates through internal research programs, which could materially adversely affect our future growth and prospects.

Our product candidates are all designed to treat patients with glaucoma, and the success or failure of any one of our product candidates could impact sales of our other potential products in the future.

Our product candidates are designed to be once-daily dosed ROCK inhibitor eye drops to be applied topically to lower IOP for the treatment of glaucoma through various MOAs. Accordingly, increased sales for one of our potential products may negatively impact sales for our other potential products. Our commercialization strategy is unique for each of our product candidates. However, we cannot guarantee that cannibalization of sales among our potential product lines will not occur in the future. Because each of our product candidates are ROCK inhibitor eye drops designed to treat patients with glaucoma, any challenges or failures with respect to any of these potential products could negatively impact sales or the public perception of our other potential products.

Risks Related to Our Financial Position and Need for Additional Capital

We currently have no source of revenue and may never become profitable.

We are a clinical-stage pharmaceutical company with a limited operating history. Our ability to generate revenue and become profitable depends upon our ability to successfully complete the development of our product candidates for the management of glaucoma and obtain the necessary regulatory approvals for our product candidates. We have never been profitable, have no products approved for commercial sale and to date have not generated any revenue from product sales. Even if we receive regulatory approval for our products for commercial sale, we do not know when such potential products will generate revenue, if at all. Our ability to generate product revenue depends on a number of factors, including our ability to:

- successfully complete clinical development, and receive regulatory approval, for our product candidates;
- set an acceptable price for our potential products and obtain adequate reimbursement from third-party payors;
- obtain commercial quantities of our potential products at acceptable cost levels; and
- successfully market and sell our potential products in the United States and abroad.

In addition, because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses, or when, or if, we will be able to achieve or maintain profitability. In addition, our expenses could increase beyond expectations if we are required by the FDA or other regulatory authorities to perform studies in addition to those that we currently anticipate. Even if our product candidates are approved for commercial sale, we anticipate incurring significant costs associated with the commercial launch of these products.

Our ability to become and remain profitable depends on our ability to generate revenue. Even if we are able to generate revenues from the sale of our potential products, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations. Even 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 our company and could impair our ability to raise capital, expand our business or continue our operations.

We have incurred net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future.

We have incurred losses in each year since our inception in June 2005. Our net losses were \$48.1 million, \$31.1 million and \$15.0 million for the years ended December 31, 2014, 2013 and 2012, respectively. As of December 31, 2014, we had an accumulated deficit of \$143.2 million.

Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable.

Table of Contents

We have devoted the majority of our financial resources to research and development, including our non-clinical development activities and clinical trials. To date, we have financed our operations primarily through the sale of equity securities and issuance of convertible debt, including the completion of our IPO in October 2013 and the issuance of the 2014 Convertible Notes in September 2014. Our product candidates will require the completion of regulatory review, significant marketing efforts and substantial investment before they can provide us with any revenue.

We expect our research and development expenses to continue to be significant in connection with our ongoing and planned Phase 3 clinical trials. In addition, if we obtain regulatory approval for our product candidates, we expect to incur increased sales and marketing expenses. As a result, we expect to continue to incur significant and increasing operating losses and negative cash flows for the foreseeable future. These losses have had and will continue to have a material adverse effect on our stockholders' equity (deficit), financial position, cash flows and working capital.

We may need to obtain additional financing to fund our operations and, if we are unable to obtain such financing, we may be unable to complete the development and commercialization of our primary product candidates.

Our operations have consumed substantial amounts of cash since inception. In October 2013, we received net proceeds from our IPO of approximately \$68.3 million, after deducting underwriting discounts and commissions and expenses. Additionally, on September 30, 2014, the Company issued \$125.0 million aggregate principal amount of the 2014 Convertible Notes. The Company received net proceeds from the issuance of the 2014 Convertible Notes of approximately \$124.1 million, after deducting discounts and certain expenses of \$875,000. We may need to obtain additional financing to fund our future operations. Additionally, we may need to obtain additional financing to conduct additional trials for the approval of our drug candidates if requested by regulatory bodies, and completing the development of any additional product candidates we might acquire. Moreover, our fixed expenses such as rent and other contractual commitments are substantial and are expected to increase in the future.

Our future funding requirements will depend on many factors, including, but not limited to:

- the progress, timing, scope and costs of our clinical trials, including the ability to timely enroll patients in our planned and potential future clinical trials;
- the time and cost necessary to obtain regulatory approvals that may be required by regulatory authorities;
- our ability to successfully commercialize our product candidates;
- the amount of sales and other revenues from product candidates that we may commercialize, if any, including the selling prices for such potential products and the availability of adequate third-party reimbursement;
- selling and marketing costs associated with our potential products, including the cost and timing of expanding our marketing and sales capabilities;
- the terms and timing of any potential future collaborations, licensing or other arrangements that we may establish;
- cash requirements of any future acquisitions and/or the development of other product candidates;
- the costs of operating as a public company;
- the time and cost necessary to respond to technological and market developments; and
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

Until we can generate a sufficient amount of revenue, we may finance future cash needs through public or private equity offerings, license agreements, debt financings, collaborations, strategic alliances and marketing or distribution arrangements. Additional funds may not be available when we need them on terms that are acceptable to us, or at all. If adequate funds are not available, we may be required to delay or reduce the scope of or eliminate one or more of our research or development programs or our commercialization efforts. We may seek to access the public or private capital markets whenever conditions are favorable, even if we do not have an immediate need for additional capital at that time. In addition, if we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or to grant licenses on terms that may not be favorable to us.

Table of Contents

We believe that our existing cash and cash equivalents and investments will be sufficient to fund our operations through product commercialization, which is expected in 2017. However, until we can generate a sufficient amount of revenue, we may be required to obtain further funding through other public or private offerings, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts. Our forecast of the period of time through which our financial resources will be adequate to support our operating requirements is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed elsewhere in this “Risk Factors” section. We have based this estimate on a number of assumptions that may prove to be wrong, and changing circumstances beyond our control may cause us to consume capital more rapidly than we currently anticipate. Our inability to obtain additional funding when we need it could seriously harm our business.

Our substantial leverage and related obligations could adversely affect our financial condition and restrict our operating flexibility.

We have substantial debt and related obligations. As of December 31, 2014, our total indebtedness consisted of our \$125.0 million aggregate principal amount of 2014 Convertible Notes. Our substantial level of debt and related obligations, including interest payments, covenants and restrictions, could have important consequences, including the following:

- impairing our ability to successfully complete the development of our product candidates, which would prevent us from generating a source of revenue and becoming profitable;
- making it more difficult for us to satisfy our obligations with respect to our indebtedness, which could result in an event of default under the agreement governing the 2014 Convertible Notes;
- limiting our ability to obtain additional financing on satisfactory terms to fund our working capital requirements, capital expenditures, acquisitions, debt obligations and other general corporate requirements;
- increasing our vulnerability to general economic downturns, competition and industry conditions, which could place us at a competitive disadvantage compared to our competitors that are less leveraged and therefore we may be unable to take advantage of opportunities that our leverage prevents us from exploiting; and
- imposing additional restrictions on the manner in which we conduct our business, including restrictions on our ability to pay dividends, incur additional debt and sell assets.

The occurrence of any one of these events could have an adverse effect on our business, financial condition, operating results or cash flows and ability to satisfy our obligations under our indebtedness.

Although the agreement governing the 2014 Convertible Notes contains restrictions on the incurrence of additional indebtedness, these restrictions are subject to a number of significant qualifications and exceptions, and any indebtedness incurred in compliance with these restrictions could be substantial. In addition, the agreement governing the 2014 Convertible Notes allows us to incur a significant amount of indebtedness in connection with acquisitions and a significant amount of purchase money debt. If new debt is added to current debt levels, the related risks that we and noteholders face would be increased.

The terms of the agreement governing the 2014 Convertible Notes may restrict our current and future operations, particularly our ability to respond to changes in our business or to take certain actions.

The agreement governing the 2014 Convertible Notes contains, and the terms of any future indebtedness of ours would likely contain, a number of restrictive covenants that impose significant operating restrictions, including restrictions on our ability to engage in acts that may be in our best long-term interests. The agreement governing the 2014 Convertible Notes includes covenants that, among other things, restrict or otherwise limit our ability to:

- incur additional indebtedness and create liens;
- pay dividends on capital stock and make other restricted payments;
- enter into any merger, partnership, joint venture, syndicate, pool, profit-sharing or royalty agreement, or engage in any transactions with our affiliates;

sell or transfer assets;
merge; and

43

Table of Contents

issue equity securities senior to our common stock or convertible or exercisable for equity securities senior to our common stock.

If not cured, as applicable, a breach of any of these provisions could result in a default under the agreement governing the 2014 Convertible Notes that would allow noteholders to declare the outstanding debt immediately due and payable. In addition, the 2014 Convertible Notes are secured by substantially all of our existing and hereafter created or acquired assets, including our intellectual property, accounts receivable, equipment, general intangibles, inventory and investment property, and all of the proceeds and products of the foregoing. If we are unable to pay those amounts because we do not have sufficient cash on hand or are unable to obtain alternative financing on acceptable terms, the noteholders could initiate a bankruptcy proceeding or proceed against any assets that serve as collateral to secure the 2014 Convertible Notes.

These restrictions could limit our ability to obtain future financings, make needed capital expenditures, withstand future downturns in the economy or otherwise conduct necessary corporate activities. We may also be prevented from taking advantage of business opportunities that arise because of limitations imposed on us by the restrictive covenants under the 2014 Convertible Notes.

We may sell additional equity or debt securities to fund our operations, which may result in dilution to our stockholders and impose restrictions on our business.

In order to raise additional funds to support our operations, we may sell additional equity or debt securities, which would result in dilution to all of our stockholders or impose restrictive covenants that adversely impact our business. The incurrence of indebtedness would result in increased fixed payment obligations and could also result in restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we are unable to expand our operations or otherwise capitalize on our business opportunities, our business, financial condition and results of operations could be materially adversely affected.

Our short operating history may make it difficult for investors to evaluate the success of our business to date and to assess our future viability.

We are a clinical-stage company. We were incorporated and commenced active operations in the second quarter of 2005. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital and developing our product candidates. We have not yet demonstrated our ability to successfully complete a Phase 3 registration trial, obtain regulatory approval, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a product development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Risks Related to Our Reliance on Third Parties

We have no manufacturing capacity and anticipate continued reliance on third-party manufacturers for the development and commercialization of our product candidates in accordance with manufacturing regulations.

We do not currently operate manufacturing facilities for clinical or commercial production of our product candidates. We have no experience in drug formulation, and we lack the resources and the capabilities to manufacture our product candidates and potential products on a clinical or commercial scale. We do not intend to develop facilities for the manufacture of product candidates and potential products for clinical trials or commercial purposes in the foreseeable future. We currently rely on third-party manufacturers to produce the active pharmaceutical ingredient and final drug product for our clinical trials. We manage such production with all our vendors on a purchase order basis in accordance with applicable master service and supply agreements. We do not have long-term agreements with any of these or any other third-party suppliers to support our clinical trials. To the extent we terminate our existing supplier arrangements in the future and seek to enter into arrangements with alternative suppliers, we might experience a delay in our ability to obtain our commercial supplies.

With respect to production of our potential commercial products in the future, we plan on outsourcing production of the active pharmaceutical ingredients and final product manufacturing if and when approved for marketing by the applicable regulatory authorities. We have entered into a contractual relationship for the final commercial drug product manufacturing. However, we do not have any current contractual relationships for the commercial production of the active pharmaceutical ingredients. This

44

Table of Contents

process is difficult and time consuming and we can give no assurance that we will enter any future commercial supply agreements with any contract manufacturers on favorable terms or at all.

Reliance on third-party manufacturers entails risks, including:

- manufacturing delays if our third-party manufacturers give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of their agreements with us;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- the possible breach of the manufacturing agreement by the third party;
- product loss due to contamination, equipment failure or improper installation or operation of equipment or operator error;
- the failure of the third-party manufacturer to comply with applicable regulatory requirements; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

Our manufacturers may not perform as agreed or may not remain in the contract manufacturing business. In the event of a natural disaster, business failure, strike or other difficulty, we may be unable to replace a third-party manufacturer in a timely manner and the production of our product candidates and potential products could be interrupted, resulting in delays and additional costs. We may also have to incur other charges and expenses for products that fail to meet specifications and undertake remediation efforts.

If third-party manufacturers fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.

Before a third party can begin commercial manufacture of our product candidates and potential products, contract manufacturers must obtain regulatory approval of their manufacturing facilities, processes and quality systems. Due to the complexity of the processes used to manufacture pharmaceutical products and product candidates, any potential third-party manufacturer may be unable to initially pass federal, state or international regulatory inspections in a cost effective manner. If contract manufacturers are not approved by the FDA, our commercial supply of drug substance will be significantly delayed and may result in significant additional costs.

In addition, pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and foreign regulatory authorities, before and after product approval, and must comply with cGMP. Our contract manufacturers may encounter difficulties in achieving quality control and quality assurance and may experience shortages in qualified personnel. In addition, contract manufacturers' failure to achieve and maintain high manufacturing standards in accordance with applicable regulatory requirements, or the incidence of manufacturing errors, could result in patient injury, product liability claims, product shortages, product recalls or withdrawals, delays or failures in product testing or delivery, cost overruns or other problems that could seriously harm our business. If a third-party manufacturer with whom we contract is unable to comply with manufacturing regulations, we may also be subject to fines, unanticipated compliance expenses, recall or seizure of our products, product liability claims, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions could materially adversely affect our financial results and financial condition.

Furthermore, changes in the manufacturing process or procedure, including a change in the location where the product is manufactured or a change of a third-party manufacturer, will require prior FDA review and/or approval of the manufacturing process and procedures in accordance with the FDA's regulations, or comparable foreign requirements. This review may be costly and time consuming and could delay or prevent the launch of a product. The new facility will also be subject to pre-approval inspection. In addition, we have to demonstrate that the product made at the new facility is equivalent to the product made at the former facility by physical and chemical methods, which are costly and time consuming. It is also possible that the FDA may require clinical testing as a way to prove equivalency, which would result in additional costs and delay.

Table of Contents

Any collaboration arrangement that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our current and potential future product candidates.

We may seek collaboration arrangements with pharmaceutical or biotechnology companies for the development or commercialization of our current and potential future product candidates. We will face, to the extent that we decide to enter into collaboration agreements, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, document and implement. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we choose to enter into such arrangements, and the terms of the arrangements may not be favorable to us. If and when we collaborate with a third party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third party. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations.

Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

We currently depend on third parties to conduct some of the operations of our clinical trials and other portions of our operations, and we may not be able to control their work as effectively as if we performed these functions ourselves. We rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to oversee and conduct our clinical trials, and to perform data collection and analysis of our product candidates. We expect to rely on these third parties to conduct clinical trials of any other potential products that we develop. These parties are not our employees and we cannot control the amount or timing of resources that they devote to our program. In addition, any CRO that we retain will be subject to the FDA's regulatory requirements or similar foreign standards and we do not have control over compliance with these regulations by these providers. Our agreements with third-party service providers are on a trial-by-trial and project-by-project bases. Typically, we may terminate the agreements with notice and are responsible for the third party's incurred costs. If any of our relationships with our third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or regulatory approval of our product candidates or commercialization of our potential products, producing additional losses and depriving us of potential product revenue.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities, and we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan, the protocols for the trial and the FDA's regulations and international standards, referred to as GCP requirements, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Preclinical studies must also be conducted in compliance with the Animal Welfare Act requirements. Managing performance of third-party service providers can be difficult, time consuming and cause delays in our development programs. We currently have a small number of employees, which limits the internal resources we have available to identify and monitor our third-party providers.

Furthermore, these third parties may produce or manufacture competing drugs or may have relationships with other entities, some of which may be our competitors. The use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. If these third parties do not successfully carry out their contractual duties or obligations and meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the

failure to adhere to our clinical protocols according to regulatory requirements or for other reasons, our financial results and the commercial prospects for our current product candidates or our other potential product candidates could be harmed, our costs could increase and our ability to obtain regulatory approval and commence product sales could be delayed.

Table of Contents

If we fail to establish an effective distribution process our business may be adversely affected.

We do not currently have the infrastructure necessary for distributing pharmaceutical products to patients. We intend to contract with third-party logistics wholesalers to warehouse these products and distribute them to pharmacies. This distribution network will require significant coordination with our sales and marketing and finance organizations. Failure to secure contracts with wholesalers could negatively impact the distribution of our products, and failure to coordinate financial systems could negatively impact our ability to accurately report product revenue. If we are unable to effectively establish and manage the distribution process, the commercial launch and sales of our products will be delayed or severely compromised and our results of operations may be harmed.

Risks Related to Intellectual Property

We may not be able to protect our proprietary technology in the marketplace.

We depend on our ability to protect our proprietary technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability and any future licensee's ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We believe we will be able to obtain, through prosecution of our current pending patent applications, adequate patent protection for our proprietary drug technology. If we are compelled to spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing or acquiring, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed. If we are unable to effectively protect the intellectual property that we own, other companies may be able to offer the same or similar products for sale, which could materially adversely affect our competitive business position and harm our business prospects. Our patents may be challenged, narrowed, invalidated, or circumvented, which could limit our ability to stop competitors from marketing the same or similar products or limit the length of term of patent protection that we may have for our products.

The patent positions of pharmaceutical products are often complex and uncertain. The breadth of claims allowed in pharmaceutical patents in the United States and many jurisdictions outside of the United States is not consistent. For example, in many jurisdictions the support standards for pharmaceutical patents are becoming increasingly strict. Some countries prohibit method of treatment claims in patents. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or create uncertainty. In addition, publication of information related to our current product candidates and potential products may prevent us from obtaining or enforcing patents relating to these product candidates and potential products, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

Our intellectual property includes issued patents and pending patent applications for compositions of matter and methods of use. As of December 31, 2014, we own 9 patents and have 11 patent applications in the United States and certain foreign jurisdictions for our primary product candidates Rhopressa™ and Roclatan™. Patent protection for Roclatan™ arises from the U.S. patents that cover Rhopressa™. The patents cover composition of matter and method of use. We own 35 patents and have 11 pending patent applications in the United States and certain foreign jurisdictions relating to our previously discontinued product candidates and other proprietary technology. See "Business—Intellectual Property" included elsewhere in this report for further information about our issued patents and patent applications. Patents that we own or may license in the future do not necessarily ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

- our patents may not be broad or strong enough to prevent competition from other products that are identical or similar to our product candidates;
- there can be no assurance that the term of a patent can be extended under the provisions of patent term extension afforded by U.S. law or similar provisions in foreign countries, where available;
- our issued patents and patents that we may obtain in the future may not prevent generic entry into the market for our Rhopressa™ and Roclatan™ product candidates;

- we do not at this time own or control issued foreign patents outside of Europe that would prevent generic entry into those markets for our product candidates;
- we may be required to disclaim part of the term of one or more patents;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim;

Table of Contents

- there may be prior art of which we are aware, which we do not believe affects the validity or enforceability of a patent claim, but which, nonetheless, ultimately may be found to affect the validity or enforceability of a patent claim;
- there may be other patents issued to others that will affect our freedom to operate;
- if our patents are challenged, a court could determine that they are invalid or unenforceable;
- there might be a significant change in the law that governs patentability, validity and infringement of our patents that adversely affects the scope of our patent rights;
 - a court could determine that a competitor's technology or product does not infringe our patents;
 - and
- our patents could irretrievably lapse due to failure to pay fees or otherwise comply with regulations or could be subject to compulsory licensing.

If we encounter delays in our development or clinical trials, the period of time during which we could market our potential products under patent protection would be reduced.

Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which our competitors claim that our patents are invalid, unenforceable and/or not infringed.

Alternatively, our competitors may seek approval to market their own products similar to or otherwise competitive with our products. In these circumstances, we may need to defend and/or assert our patents, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid and/or unenforceable. We may also fail to identify patentable aspects of our research and development before it is too late to obtain patent protection. Even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

The issuance of a patent is not conclusive as to its inventorship, scope, ownership, priority, validity or enforceability. In that regard, third parties may challenge our patents in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and potential products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

A significant portion of our intellectual property portfolio currently comprises pending patent applications that have not yet been issued as granted patents. If our pending patent applications fail to issue our business will be adversely affected.

Our commercial success will depend significantly on maintaining and expanding patent protection for our product candidates, as well as successfully defending our current and future patents against third-party challenges. As of December 31, 2014, we own 44 patents and have 22 pending patent applications in the United States and certain foreign jurisdictions relating to our current and previously discontinued product candidates and proprietary technology. See "Business—Intellectual Property" included elsewhere in this report for further information about our issued patents and patent applications. Our issued patents include 9 patents for composition of matter and method of use covering our lead product candidate, Rhopressa™ in the United States and certain foreign jurisdictions. These patents also cover our other primary product candidate Roclatan™ to the extent that Rhopressa™ forms a part of Roclatan™. The remainder of our portfolio is made up of patents covering previously discontinued product candidates and other proprietary technology and pending patent applications that have not yet been issued by the USPTO, or any other jurisdiction that cover our current and previously discontinued product candidates or other proprietary technology. There can be no assurance that our pending patent applications will result in issued patents in the United States or foreign jurisdictions in which such applications are pending. Even if patents do issue on any of these applications,

there can be no assurance that a third party will not challenge their validity or that we will obtain sufficient claim scope in those patents to prevent a third party from competing successfully with our products.

We may not be able to enforce our intellectual property rights throughout the world.

The laws of some foreign countries do not protect intellectual property rights to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in

Table of Contents

certain foreign jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property protection, especially those relating to life sciences. It may be difficult for us to stop the infringement of our patents or the misappropriation of these intellectual property rights in any foreign jurisdictions. For example, some foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, many countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit.

Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate. In addition, changes in the law and legal decisions by courts in the United States and foreign countries may affect our ability to obtain adequate protection for our technology and the enforcement of intellectual property.

We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing our products.

Our commercial success depends significantly on our ability to operate without infringing the patents and other intellectual property rights of third parties. For example, there could be issued patents of which we are not aware that our product candidates or potential products infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe.

Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our product candidates or potential products infringe. For example, pending applications may exist that claim or can be amended to claim subject matter that our product candidates or potential products infringe. Competitors may file continuing patent applications claiming priority to already issued patents in the form of continuation, divisional, or continuation-in-part applications, in order to maintain the pendency of a patent family and attempt to cover our product candidates.

Third parties may assert that we are employing their proprietary technology without authorization and may sue us for patent or other intellectual property infringement. These lawsuits are costly and could adversely affect our results of operations and divert the attention of managerial and scientific personnel. If we are sued for patent infringement, we would need to demonstrate that our product candidates, potential products or methods either do not infringe the claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion. If a court holds that any third-party patents are valid, enforceable and cover our products or their use, the holders of any of these patents may be able to block our ability to commercialize our products unless we acquire or obtain a license under the applicable patents or until the patents expire. We may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost or on reasonable terms. Any inability to secure licenses or alternative technology could result in delays in the introduction of our products or lead to prohibition of the manufacture or sale of products by us. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, in any such proceeding or litigation, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade

secrets could have a similar negative impact on our business. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

We may be subject to claims that we or our employees have misappropriated the intellectual property, including trade secrets, of a third party, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities, biotechnology companies or other pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements in connection with such previous employment. Although we try to ensure that our employees do not use the intellectual property and other proprietary

Table of Contents

information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed such intellectual property, including trade secrets or other proprietary information. Litigation may be necessary to defend against these claims. We are not aware of any threatened or pending claims related to these matters or concerning the agreements with our senior management, but litigation may be necessary in the future to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our management and scientific personnel.

We may be unable to adequately prevent disclosure of trade secrets and other proprietary information.

We rely on trade secrets to protect our proprietary know-how and technological advances, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. However, any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets. Accordingly, these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights. In addition, others may independently discover our trade secrets and proprietary information. Further, the FDA, as part of its Transparency Initiative, a proposal by the FDA to increase disclosure and make data more accessible to the public, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Failure to obtain or maintain trade secret protection could enable competitors to use our proprietary information to develop products that compete with our products or cause additional, material adverse effects upon our competitive business position and financial results. Any lawsuits relating to infringement of intellectual property rights brought by or against us will be costly and time consuming and may adversely impact the price of our common stock.

We may be required to initiate litigation to enforce or defend our intellectual property. These lawsuits can be very time consuming and costly. There is a substantial amount of litigation involving patent and other intellectual property rights in the pharmaceutical industry generally. Such litigation or proceedings could substantially increase our operating expenses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

In any infringement litigation, any award of monetary damages we receive may not be commercially valuable. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are resolved. Further, any claims we assert against a perceived infringer could provoke these parties to assert counterclaims against us alleging that we have infringed their patents. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

In addition, our patents and patent applications could face other challenges, such as interference proceedings, opposition proceedings, re-examination proceedings, and other forms of post-grant review. In the United States, for example, post-grant review has recently been expanded. Any of these challenges, if successful, could result in the invalidation of, or in a narrowing of the scope of, any of our patents and patent applications subject to challenge. Any of these challenges, regardless of their success, would likely be time consuming and expensive to defend and resolve and would divert our management and scientific personnel's time and attention. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the market price of our common stock.

Table of Contents

We will need to obtain FDA approval of any proposed product names, and any failure or delay associated with such approval may adversely affect our business.

We assigned the trade names Rhopressa™ and Roclatan™ to our lead product candidates in 2014, with trademark applications for registration pending from the USPTO. These and any other names we intend to use for our product candidates will require approval from the FDA regardless of whether we have secured a formal trademark registration from the USPTO. The FDA typically conducts a review of proposed product names, including an evaluation of the potential for confusion with other product names. The FDA may also object to a product name if it believes the name inappropriately implies medical claims or contributes to an overstatement of efficacy. If the FDA objects to any of our proposed product names, we may be required to adopt an alternative name for our product candidates. If we adopt an alternative name, we would lose the benefit of our existing trademark applications for such product candidate and may be required to expend significant additional resources in an effort to identify a suitable product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. We may be unable to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product candidates.

If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation extending the terms of our patents and obtaining data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA regulatory approval for our product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval by the FDA.

The application for patent term extension is subject to approval by the USPTO, in conjunction with the FDA. It takes at least six months to obtain approval of the application for patent term extension. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain earlier approval of competing products, and our ability to generate revenues could be materially adversely affected.

Risks Related to Our Business Operations and Industry

We depend upon our key personnel and our ability to attract and retain employees.

Our future growth and success depend on our ability to recruit, retain, manage and motivate our employees. We are highly dependent on our senior management team and our scientific founders, as well as the other principal members of our management and scientific teams. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from terminating their employment with us at any time. The loss of the services of any member of our senior management or scientific team or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of Vicente Anido, Jr., our Chairman of the Board of Directors and Chief Executive Officer, Thomas A. Mitro, our President and Chief Operating Officer, Richard J. Rubino, our Chief Financial Officer, Brian Levy, our Chief Medical Officer or Casey C. Kopczynski, our Chief Scientific Officer, could be detrimental to us if we cannot recruit suitable replacements in a timely manner. We do not currently carry “key person” insurance on the lives of members of executive management. The competition for qualified personnel in the pharmaceutical field is intense. Due to this intense competition, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to

recruit suitable replacement personnel. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

As a public company, we are subject to the periodic reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under

Table of Contents

the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

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 an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

We will need to significantly increase the size of our organization, and we may experience difficulties in managing growth.

We are currently a small company with 40 full-time employees as of December 31, 2014. In order to commercialize our potential products, we will need to substantially increase our operations. We plan to continue to build our compliance, financial and operating infrastructure to ensure the maintenance of a well-managed company. We expect to expand our employment base to approximately 300 when we are in the full commercial stages of our current potential products' life cycle.

Future growth will impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees. In addition, to meet our obligations as a public company, we will need to increase our general and administrative capabilities. Our management, personnel and systems currently in place may not be adequate to support this future growth. Our future financial performance and our ability to commercialize our potential products and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to:

- manage our clinical trials and the regulatory process effectively;
- manage the manufacturing of product candidates and potential products for clinical and commercial use;
- integrate current and additional management, administrative, financial and sales and marketing personnel;
- develop a marketing and sales infrastructure;
- hire new personnel necessary to effectively commercialize our product candidates;
- develop our administrative, accounting and management information systems and controls; and
- hire and train additional qualified personnel.

Product candidates that we may acquire or develop in the future may be intended for patient populations that are large. In order to continue development and marketing of these product candidates, if approved, we would need to significantly expand our operations. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third parties.

If we engage in acquisitions or licenses in the future, we will incur a variety of costs and we may never realize the anticipated benefits of such acquisitions or licenses.

We may attempt to acquire or license businesses, technologies, services, products or product candidates in the future that we believe are a strategic fit with our business. We have no present agreement regarding any material acquisitions or licenses. However, if we do undertake any acquisitions or licenses, the process of integrating an acquired or licensed business, technology, service, product or product candidate into our business may result in unforeseen operating difficulties and expenditures, including diversion of resources and management's attention from our core business. In addition, we may fail to retain key executives and employees of the companies we acquire, which may reduce the value of the acquisition or give rise to additional integration costs. Future acquisitions or licenses could result in additional issuances of equity securities that would dilute the ownership of existing stockholders. Future acquisitions or licenses could also result in the incurrence of debt, actual or contingent liabilities or the amortization of expenses related to other intangible assets, any of which could adversely affect our operating results.

We have limited experience identifying, negotiating and implementing acquisitions or licenses of additional product candidates, which is a lengthy and complex process. The market for acquiring or licensing product candidates is intensely competitive, and other companies, including some with substantially greater financial, marketing and sales resources, may also pursue strategies

Table of Contents

to acquire or license products, product candidates or technologies that we may consider attractive. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us.

We have limited resources to identify and execute the acquisition or licensing of additional product candidates and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire or license the rights to additional product candidates on terms that we find acceptable, or at all. Any product candidate that we acquire or license may require additional development efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities

Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases to patients. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations.

Interest rates and the ability to access credit markets could also adversely affect the ability of patients, payors and distributors to purchase, pay for and effectively distribute our products. Similarly, these macroeconomic factors could affect the ability of our potential future contract manufacturers, sole-source or single-source suppliers or licensees to remain in business or otherwise manufacture or supply product. Failure by any of them to remain in business could affect our ability to manufacture products.

If product liability lawsuits are successfully brought against us, our insurance may be inadequate and we may incur substantial liability.

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates. We will face an even greater risk if we commercially sell our potential products or any other product candidate that we develop. We maintain primary product liability insurance and excess product liability insurance that cover our clinical trials, and we plan to maintain insurance against product liability lawsuits for commercial sale of our potential products. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and, in the future, commercial use of our potential products, for which our insurance coverage may not be adequate, and the cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial.

For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated adverse effects. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;

- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- initiation of investigations by regulators;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;

Table of Contents

- significant costs to defend resulting litigation;
- diversion of management and scientific resources from our business operations;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We will need to increase our insurance coverage if and when we begin selling our product candidates if and when they receive marketing approval. However, the product liability insurance we will need to obtain in connection with the commercial sales of our product candidates if and when they receive regulatory approval may be unavailable in meaningful amounts or at a reasonable cost. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates if and when they obtain regulatory approval, which could materially adversely affect our business, financial condition, results of operations, cash flows and prospects.

Additionally, we do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, employment practices liability, property, auto, workers' compensation, products liability and directors' and officers' insurance. We do not know, however, if we will be able to maintain insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would materially adversely affect our financial position, cash flows and results of operations.

Business interruptions could delay us in the process of developing our products and could disrupt our sales.

Our principal executive offices are located in Irvine, California, our clinical and finance operations are located in Bedminster, New Jersey and our research and development facility is located in Research Triangle Park, North Carolina. We are vulnerable to natural disasters, such as severe storms and other events that could disrupt our operations. We do not carry insurance for natural disasters and we may not carry sufficient business interruption insurance to compensate us for losses that may occur. Any losses or damages we incur could have a material adverse effect on our business operations.

Our business and operations would suffer in the event of system failures.

Despite the implementation of security measures, our internal computer systems, and those of our CROs and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading, which could significantly harm our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We adopted a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or

unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Table of Contents

Risks Related to Ownership of Our Common Stock

The market price of our common stock has been, and may continue to be highly volatile.

Our stock price has been volatile and is likely to continue to be volatile. Since shares of our common stock were sold in our IPO in October 2013 at a price of \$10.00 per share, our closing stock price has reached a high of \$31.25 and a low of \$10.40, through February 20, 2015. The following factors, in addition to other factors described in this “Risk Factors” section, may have a significant impact on the market price of our common stock:

- the results of our testing and clinical trials, including the results of our Phase 3 registration trial for Rhopressa™ and the results of our planned Phase 3 registration trials for Roclatan™;
- announcements of regulatory approval or a complete response letter, or specific label indications or patient populations for its use, or changes or delays in the regulatory review process;
- announcements of therapeutic innovations or new products by us or our competitors;
- adverse actions taken by regulatory agencies with respect to our clinical trials, manufacturing supply chain or sales and marketing activities;
- any adverse changes to our relationship with manufacturers or suppliers;
- the results of our efforts to acquire or license additional product candidates;
- variations in the level of expenses related to our existing product candidates or preclinical and clinical development programs;
- any intellectual property infringement actions in which we may become involved;
- announcements concerning our competitors or the pharmaceutical industry in general;
- achievement of expected product sales and profitability;
- manufacture, supply or distribution shortages;
- actual or anticipated fluctuations in our quarterly or annual operating results;
- changes in financial estimates or recommendations by securities analysts;
- trading volume of our common stock;
- sales of our common stock by us, our executive officers and directors or our stockholders in the future;
- general economic and market conditions and overall fluctuations in the U.S. equity markets;
- changes in accounting principles; and
- the loss of any of our key scientific or management personnel.

In addition, the stock market, in general, and small pharmaceutical and biotechnology companies have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. Further, the current decline in the financial markets and related factors beyond our control may cause our stock price to decline rapidly and unexpectedly.

We may be subject to securities litigation, which is expensive and could divert management attention.

Our stock price has been volatile, and in the past companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Litigation of this type could result in substantial costs and diversion of management’s attention and resources, which could adversely impact our business. Any adverse determination in litigation could also subject us to significant liabilities.

Our existing principal stockholders, executive officers and directors own a significant percentage of our common stock and will be able to exert a significant control over matters submitted to our stockholders for approval.

Our officers and directors, and stockholders who own more than 5% of our outstanding common stock, beneficially own approximately 69.41% of our common stock as of December 31, 2014. This significant concentration of share ownership may adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders. As a result, these stockholders, if they acted together, could significantly influence all

Table of Contents

matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. These stockholders may be able to determine all matters requiring stockholder approval. The interests of these stockholders may not always coincide with our interests or the interests of other stockholders.

This may also prevent or discourage unsolicited acquisition proposals or offers for our common stock that other stockholders may feel are in their best interest, and our principal stockholders may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

Additionally, our amended and restated certificate of incorporation renounces any interest or expectancy that we have in, or in being offered an opportunity to participate in, corporate opportunities that are presented to our existing principal investors, their affiliates and their partners, members, directors, stockholders, employees or agents (whether or not any such person is our director), other than someone who is our employee, except that we do not renounce our interest in any corporate opportunity offered to any such person if such opportunity is offered to such person expressly and solely in his or her capacity as our director. These provisions will apply even if the opportunity is one that we might reasonably have pursued or had the ability or desire to pursue if granted the opportunity to do so.

Sales of a substantial number of shares of our common stock in the public market by our existing stockholders could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock.

If securities or industry analysts do not publish or cease publishing research or reports about us, our business or our market, or if they adversely change their recommendations or publish negative reports regarding our business or our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts may publish about us, our business, our market or our competitors. We do not have any control over these analysts and we cannot provide any assurance that analysts will cover us or provide favorable coverage. If any of the analysts who may cover us adversely change their recommendation regarding our stock, or provide more favorable relative recommendations about our competitors, our stock price could decline. If any analyst who may cover us were to cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Because we do not intend to declare cash dividends on our shares of common stock in the foreseeable future, stockholders must rely on appreciation of the value of our common stock for any return on their investment. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends in the foreseeable future. In addition, the terms of the 2014 Convertible Notes and any future debt agreements may preclude us from paying dividends. As a result, we expect that only appreciation of the price of our common stock, if any, will provide a return to investors for the foreseeable future.

Our ability to use our net operating loss carry-forwards may be limited.

As of December 31, 2014, we had federal net operating losses of approximately \$133.6 million, which may be utilized against future federal income taxes. These net operating losses will begin to expire at various dates beginning in 2024, if not utilized. If we experience an "ownership change" for purposes of Section 382 of the Internal Revenue Code of 1986, as amended, we may be subject to annual limits on our ability to utilize net operating loss carry-forwards. An ownership change is, as a general matter, triggered by sales or acquisitions of our stock in excess of 50% on a cumulative basis during a three-year period by persons owning 5% or more of our total equity value. We are not currently subject to any annual limits on our ability to utilize net operating loss carry-forwards. Our deferred tax assets have been fully offset by a valuation allowance as of December 31, 2014.

The requirements associated with being a public company require significant company resources and management attention.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, the listing requirements of the securities exchange on which our common stock is traded, and other

56

Table of Contents

applicable securities rules and regulations. The Exchange Act requires that we file annual, quarterly and current reports with respect to our business and financial condition and maintain effective disclosure controls and procedures and internal control over financial reporting. In addition, subsequent rules implemented by the SEC and NASDAQ may also impose various additional requirements on public companies. As a result, we will incur additional legal, accounting and other expenses that we did not incur as a nonpublic company, particularly after we are no longer an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. Further, the need to establish the corporate infrastructure demanded of a public company may divert management’s attention from implementing our growth strategy. We have made, and will continue to make, changes to our corporate governance standards, disclosure controls and financial reporting and accounting systems to meet our reporting obligations. However, the measures we take may not be sufficient to satisfy our obligations as a public company, which could subject us to delisting of our common stock, fines, sanctions and other regulatory action and potentially civil litigation.

The recently enacted JOBS Act will allow us to postpone the date by which we must comply with some of the laws and regulations intended to protect investors and to reduce the amount of information we provide in our reports filed with the SEC, which could undermine investor confidence in our company and adversely affect the market price of our common stock.

For so long as we remain an “emerging growth company” as defined in the JOBS Act, we may take advantage of certain exemptions from various requirements that are applicable to public companies that are not “emerging growth companies” including:

- the provisions of Section 404(b) of the Sarbanes-Oxley Act requiring that our independent registered public accounting firm provide an attestation report on the effectiveness of our internal control over financial reporting;
- the “say on pay” provisions (requiring a non-binding stockholder vote to approve compensation of certain executive officers) and the “say on golden parachute” provisions (requiring a non-binding stockholder vote to approve golden parachute arrangements for certain executive officers in connection with mergers and certain other business combinations) of the Dodd-Frank Act and some of the disclosure requirements of the Dodd-Frank Act relating to compensation of its chief executive officer; and

- the requirement to provide detailed compensation discussion and analysis in proxy statements and reports filed under the Exchange Act, and instead provide a reduced level of disclosure concerning executive compensation.

We may take advantage of these exemptions until we are no longer an “emerging growth company.” We would cease to be an “emerging growth company” upon the earliest of: (i) December 31, 2018; (ii) the last day of the first fiscal year in which our annual gross revenues are \$1 billion or more; (iii) the date on which we have, during the previous three-year period, issued more than \$1 billion in non-convertible debt securities; or (iv) as of the end of any fiscal year in which the market value of our common stock held by non-affiliates exceeded \$700 million as of the end of the second quarter of that fiscal year.

We currently intend to take advantage of some, but not all, of the reduced regulatory and reporting requirements that will be available to us so long as we qualify as an “emerging growth company.” For example, we have irrevocably elected under Section 107 of the JOBS Act not to take advantage of the extension of time to comply with new or revised financial accounting standards available under Section 102(b) of the JOBS Act. Our independent registered public accounting firm will not be required to provide an attestation report on the effectiveness of our internal control over financial reporting so long as we qualify as an “emerging growth company,” which may increase the risk that weaknesses or deficiencies in our internal control over financial reporting go undetected. Likewise, so long as we qualify as an “emerging growth company,” we may elect not to provide investors with certain information, including certain financial information and certain information regarding compensation of our executive officers, that we would otherwise have been required to provide in filings we make with the SEC, which may make it more difficult for investors and securities analysts to evaluate our company. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock, and our stock price may be more volatile and

may decline.

57

Table of Contents

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our restated certificate of incorporation and our bylaws, as well as provisions of the Delaware General Corporation Law, or DGCL, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions include:

- establishing a classified board of directors such that not all members of the board are elected at one time;
- allowing the authorized number of our directors to be changed only by resolution of our board of directors;
- limiting the removal of directors by the stockholders;
- authorizing the issuance of “blank check” preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders;
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings; and
- requiring the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal our bylaws.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management. In addition, we are subject to Section 203 of the DGCL, which generally prohibits a Delaware corporation from engaging in any of a broad range of business combinations with an interested stockholder for a period of three years following the date on which the stockholder became an interested stockholder, unless such transactions are approved by our board of directors. This provision could have the effect of delaying or preventing a change of control, whether or not it is desired by or beneficial to our stockholders.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

Our principal executive offices are currently located in Irvine, California, our clinical and finance operations are located in Bedminster, New Jersey and our research and development facility is located in Research Triangle Park, North Carolina. In January 2015, we entered into a lease agreement under which we are leasing approximately 14,500 square feet of office space in Irvine, California. Refer to Note 17 to our audited financial statements appearing elsewhere in this report for further information. Our Bedminster, New Jersey location consists of approximately 14,000 square feet of leased office space under a lease that expires in August 2020. Our research and development facility consists of approximately 5,300 square feet of leased laboratory space under an annual lease agreement. We may require additional space and facilities as our business expands.

ITEM 3. LEGAL PROCEEDINGS

We are not currently subject to any material pending legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES

None.

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 has been trading on the NASDAQ Global Market under the symbol "AERI" since our initial public offering ("IPO") on October 25, 2013. Prior to this date, there was no public market for our common stock. As a result, we have not set forth quarterly information with respect to the high and low prices for our common stock for the two most recent fiscal years.

On February 20, 2015, the closing price for our common stock as reported on the NASDAQ Global Market was \$27.71. The following table sets forth the high and low intraday sale prices per share of our common stock for the periods indicated as reported by the NASDAQ Global Market.

	High	Low
2014		
Fourth Quarter	\$32.50	\$19.46
Third Quarter	27.25	16.05
Second Quarter	29.71	13.66
First Quarter	27.15	15.03

2013

Fourth Quarter (beginning October 25, 2013)	\$18.50	\$10.25
---	---------	---------

Stockholders

As of February 20, 2015, we had 24,046,939 shares of common stock outstanding held by approximately 11 stockholders of record. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in "street" name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Table of Contents

Stock Performance Graph

The following graph illustrates a comparison of the total cumulative stockholder return on our common stock since October 25, 2013, which is the date our common stock first began trading on the NASDAQ Global Market, to two indices: the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The graph assumes an initial investment of \$100 on October 25, 2013, in our common stock and in the stocks comprising each index. It also assumes reinvestment of dividends, if any. Historical stockholder return shown is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.

*This performance graph shall not be deemed “soliciting material” or be deemed “filed” for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities under that Section, and shall not be deemed to be incorporated by reference into any of our filings under the Securities Act, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing.

Dividend Policy

We have not declared or paid any cash dividends on our capital stock in the last two fiscal years. We currently anticipate that we will retain future earnings, if any, for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends in the foreseeable future. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, we anticipate that only appreciation of the price of our common stock, if any, will provide a return to investors for at least the foreseeable future.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this report.

Recent Sales of Unregistered Securities

None.

Table of Contents

Use of Proceeds from Registered Securities

On October 30, 2013, we completed our IPO and issued 7,728,000 shares of our common stock at an IPO price of \$10.00 per share, including 1,008,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares to cover over-allotments. The shares were registered under the Securities Act on a Registration Statement on Form S-1 (Registration No. 333-191219). The SEC declared the registration statement effective on October 24, 2013.

We have invested the net proceeds from the IPO in a variety of capital preservation investments, including short-term and long-term, investment grade, interest bearing instruments. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed with the SEC on October 28, 2013 pursuant to Rule 424(b) under the Securities Act.

Table of Contents

ITEM 6. SELECTED FINANCIAL DATA

The following table sets forth our selected financial data for the periods and as of the dates indicated. You should read the following selected financial data together with the “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of this report and our audited financial statements and the accompanying notes included elsewhere in this report. We have derived the statements of operations data for the years ended December 31, 2014, 2013 and 2012 and the balance sheet data as of December 31, 2014 and 2013 from our audited financial statements included in this report. We have derived the statement of operations data for the year ended December 31, 2011 and the balance sheet data as of December 31, 2012 and 2011 from our audited financial statements not included in this report. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

	YEAR ENDED DECEMBER 31,			
	2014	2013	2012	2011
Statement of Operations and Comprehensive Loss Data:				
Expenses:				
General and administrative	\$(20,103)	\$(10,287)	\$(5,020)	\$(3,521)
Research and development	(29,869)	(11,883)	(9,273)	(10,695)
Loss from operations	(49,972)	(22,170)	(14,293)	(14,216)
Other income (expense), net	1,839	(8,978)	(685)	1,249
Net loss	\$(48,133)	\$(31,148)	\$(14,978)	\$(12,967)
Net loss attributable to common stockholders—basic and diluted ⁽¹⁾	\$(48,133)	\$(31,598)	\$(15,643)	\$(13,419)
Net loss per share attributable to common stockholders—basic and diluted ⁽¹⁾	\$(2.00)	\$(6.38)	\$(16.39)	\$(14.50)
Weighted average number of common shares outstanding—basic and diluted	24,086,651	4,955,760	954,695	925,625
	AS OF DECEMBER 31,			
	2014	2013	2012	2011
	(in thousands)			
Balance Sheet Data:				
Cash and cash equivalents	\$85,586	\$69,649	\$2,925	\$15,068
Short-term investments	54,339	—	—	—
Long-term investments	18,275	—	—	—
Total assets	161,085	70,458	3,219	15,458
Notes payable, net of discount, and accrued interest thereon	—	—	2,347	—
Warrants liability—related parties	—	—	2,456	1,098
Convertible notes, net of discounts	124,156	—	—	—
Convertible preferred stock	—	—	60,898	60,348
Total stockholders’ equity (deficit)	28,042	66,976	(63,919)	(48,697)

(1) Prior to 2014, net loss attributable to common stockholders reflects the accretion on convertible preferred stock and, where applicable, preferred stock dividends. See Note 2 to our audited financial statements appearing elsewhere in this report for an explanation of the method used to calculate the basic and diluted net loss per share

attributable to common stockholders.

Table of Contents

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following management's discussion and analysis should be read in conjunction with our audited financial statements and related notes that appear elsewhere in this Annual Report on Form 10-K. This management's discussion and analysis contains forward-looking statements that involve risks and uncertainties. Please see "Special Note Regarding Forward-Looking Statements" for additional factors relating to such statements, and see "Risk Factors" in Part I, Item 1A of this report for a discussion of certain risk factors applicable to our business, financial condition and results of operations. Past operating results are not necessarily indicative of operating results in any future periods.

Overview

We are a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with glaucoma and other diseases of the eye. Our lead product candidate, once-daily, triple-action Rhopressa™, successfully completed a Phase 2b clinical trial in patients with open-angle glaucoma and ocular hypertension in May 2013. Phase 3 registration trials commenced in July 2014. Our Phase 3 registration trial ("Rocket 1") and a second Phase 3 registration trial ("Rocket 2") will measure efficacy over three months. Rocket 2 is also designed to assess safety over 12 months. In addition, we are conducting a one year, safety-only study in Canada, named "Rocket 3." Pending successful advancement of the Phase 3 registration trials, three-month efficacy results are expected in the middle of the second-quarter 2015 for Rocket 1 and by mid-2015 for Rocket 2. Our second product candidate, once-daily, quadruple-action Roclatan™, which is a fixed-dose combination of Rhopressa™ and latanoprost, the most commonly prescribed drug for the treatment of patients with glaucoma, successfully completed a Phase 2b clinical trial in patients with open-angle glaucoma and ocular hypertension in June 2014. We expect Phase 3 registration trials to commence in mid-2015. Preparatory steps for these trials have already commenced.

We are developing Rhopressa™ as the first of a new class of compounds that is designed to lower intraocular pressure, or IOP, through novel biochemical targets. By inhibiting these targets, we believe Rhopressa™ reduces IOP via three separate mechanisms of action, or MOAs: (i) it increases fluid outflow through the trabecular meshwork, the diseased tissue of the eye, (ii) it reduces episcleral venous pressure, which represents the pressure of the blood in the episcleral veins of the eye where eye fluid drains into the bloodstream, and (iii) it reduces the production of eye fluid. Roclatan™ is a combination of Rhopressa™ and latanoprost and is designed to lower IOP through the same three MOAs as Rhopressa™ and, with a fourth MOA, through the ability of latanoprost to increase fluid outflow through the uveoscleral pathway, the eye's secondary drain.

Our mission is to build a major ophthalmic pharmaceutical company. In addition to our primary product candidates, Rhopressa™ and Roclatan™, we are also exploring the longer-term impact of Rhopressa™ and Roclatan™ on the diseased trabecular meshwork and evaluating possible uses of our existing proprietary portfolio of Rho Kinase inhibitors beyond glaucoma. We recently issued a research update on preclinical results demonstrating the potential for Rhopressa™ to have disease-modifying activity in glaucoma by stopping fibrosis in the trabecular meshwork, and also increasing perfusion in the trabecular outflow pathway thus increasing the delivery of nutrients to the diseased tissue. Additionally, an early-stage molecule, AR-13154, has shown the preclinical potential to decrease lesions in wet age-related macular degeneration at numerically higher levels than current market leading products. Our strategy includes developing our business outside of North America, including potentially obtaining clinical approval on our own for our lead compounds in Europe and possibly Japan. Regarding commercialization strategy, if our products are successful, we may potentially commercialize ourselves or with a partner in Europe, and potentially with a partner in Japan. As we prepare for foreign-based activities, we are evaluating optimized supply chain configurations and domicile alternatives for our non-U.S. intellectual property.

We may license, acquire or develop additional product candidates to broaden our presence in ophthalmology. We are continuing to explore collaboration opportunities for new ophthalmic products, delivery alternatives and new therapeutic areas, including gene therapy. However, we have no present plans, agreements or commitments with

respect to any potential acquisition, investment or license related to such additional product candidates.

We have incurred net losses since our inception in June 2005. Our operations to date have been limited to research and development and raising capital. As of December 31, 2014, we had an accumulated deficit of \$143.2 million. We recorded net losses of \$48.1 million, \$31.1 million and \$15.0 million for the years ended December 31, 2014, 2013 and 2012, respectively. We anticipate that a substantial portion of our capital resources and efforts in the foreseeable future will be focused on completing the development and obtaining regulatory approval and preparing for potential commercialization of our product candidates.

Table of Contents

We expect our research and development expenses to increase as we initiate and conduct clinical trials for our Rhopressa™ and Roclatan™ product candidates and pursue regulatory approval. As we prepare for commercialization, we will likely incur significant commercial, sales, marketing and outsourced manufacturing expenses. Since our initial public offering (“IPO”) in October 2013, we are also incurring additional expenses associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses at least for the next several years.

Prior to our IPO, we raised net cash proceeds of \$78.6 million from the private placement of \$43.8 million of convertible preferred stock and \$34.8 million of convertible notes. Subsequent to the issuance of the convertible notes, we made \$0.5 million in cash payments, \$16.2 million of the convertible notes were converted into shares of convertible preferred stock, which were subsequently converted into shares of common stock in connection with our IPO, and \$18.0 million of convertible notes initially sold in 2012 (the “2012 Convertible Notes”) were converted into shares of common stock in connection with our IPO. In connection with our IPO, all outstanding shares of convertible preferred stock were converted into shares of common stock.

On October 30, 2013, we completed our IPO and issued 7,728,000 shares of our common stock at an IPO price of \$10.00 per share, including 1,008,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares to cover over-allotments. Our shares began trading on the NASDAQ Global Market on October 25, 2013. We received net proceeds from the IPO of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million.

On September 30, 2014, we issued \$125.0 million aggregate principal amount of senior secured convertible notes (the “2014 Convertible Notes”). We received net proceeds from the issuance of the 2014 Convertible Notes of approximately \$124.1 million, after deducting discounts and certain expenses of \$875,000.

Proceeds from the 2014 Convertible Notes financing in September 2014, together with proceeds from our IPO in October 2013, are expected to provide sufficient resources to complete all currently known non-clinical and clinical requirements for our development programs advancing Rhopressa™ and Roclatan™, and approval by the U.S. Federal Drug Administration (“FDA”) and product commercialization, pending successful outcome of the trials. We also intend to use the proceeds in part for general corporate purposes and potentially for strategic growth opportunities.

On November 3, 2014, we filed a shelf registration statement on Form S-3, which was declared effective by the SEC on November 10, 2014. The shelf registration statement permits: (i) the offering, issuance and sale by us of up to a maximum aggregate offering price of \$150.0 million of our common stock; (ii) sales of common stock by certain selling stockholders; and (iii) the offering, issuance and sale of up to a maximum aggregate offering price of \$50.0 million of our common stock that may be issued and sold by us under an “at-the-market” sales agreement with Cantor Fitzgerald & Co. The common stock that may be offered, issued and sold by us under the “at-the-market” sales agreement is included in the \$150.0 million of common stock that may be offered, issued and sold by us under the shelf registration statement. As of December 31, 2014, we have not sold any securities registered pursuant to the shelf registration statement.

To date, we have not generated product revenue and we do not expect to generate product revenue unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates. If we do not successfully commercialize any of our product candidates, we may be unable to generate product revenue or achieve profitability.

We may be required to obtain further funding through public or private offerings, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate our research and development programs or commercialization efforts.

Financial Overview

Revenue

We have not generated any revenue from the sale of any products, and we do not expect to generate any revenue unless or until we obtain regulatory approval of and commercialize our products.

Table of Contents

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, benefits and stock-based compensation for all officers and employees in general management, finance and administration. Other significant expenses include facilities expenses and professional fees for accounting, legal and other services.

We expect that our general and administrative expenses will increase with the continued advancement of our product candidates and with our increased management, legal, compliance, accounting and investor relations expenses as we continue to grow. We expect these increases will likely include increased expenses for insurance, expenses related to the hiring of additional personnel and payments to outside service providers, lawyers and accountants.

Research and Development Expenses

Since our inception, we have focused on our development programs. Research and development expenses consist primarily of costs incurred for the research and development of our preclinical and clinical candidates, which include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense for research and development personnel;
- expenses incurred under agreements with contract research organizations (“CROs”), contract manufacturing organizations and service providers that conduct clinical trials and preclinical studies;
- costs associated with preclinical activities and development activities;
- costs associated with regulatory operations; and
- depreciation expense for assets used in research and development activities.

We expense research and development costs to operations as incurred. The costs for certain development activities, such as clinical trials, are recognized based on the terms of underlying agreements as well as an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations along with additional information provided to us by our vendors.

Expenses relating to activities, such as manufacturing and stability and toxicology studies, that are supportive of the product candidate itself, are classified as direct non-clinical. Expenses relating to clinical trials and similar activities, including costs associated with CROs, are classified as direct clinical. Expenses relating to activities that support more than one development program or activity such as personnel costs, stock-based compensation and depreciation are not allocated to direct clinical or non-clinical expenses and are separately classified as “unallocated.”

Table of Contents

The following table shows our research and development expenses by type of activity for the years ended December 31, 2014, 2013 and 2012:

	YEAR ENDED		
	DECEMBER 31,		
	2014	2013	2012
(in thousands)			
Rhopressa™			
Direct non-clinical	\$8,765	\$3,410	\$2,318
Direct clinical	10,994	1,607	993
Total	\$19,759	\$5,017	\$3,311
Roclatan™			
Direct non-clinical	\$690	\$481	\$—
Direct clinical	1,869	234	—
Total	\$2,559	\$715	\$—
Discontinued product candidates			
Direct non-clinical	\$89	\$596	\$2,312
Direct clinical	1	2,965	1,475
Total	\$90	\$3,561	\$3,787
Unallocated	7,461	2,590	2,175
Total research and development expense	\$29,869	\$11,883	\$9,273

For the periods presented, we did not incur any direct non-clinical or direct clinical costs for AR-13533, exploring the longer-term impact of Rhopressa™ and Roclatan™ on the diseased trabecular meshwork or evaluating possible uses of our existing proprietary portfolio of Rho Kinase inhibitors beyond glaucoma. Costs for these activities were primarily comprised of internal personnel costs and were included in unallocated costs. Discontinued product candidates relate to previously developed AR-12286 and related compounds, which did not meet their primary endpoints in clinical trials. We incurred direct non-clinical and direct clinical expenses for these discontinued product candidates in all periods presented.

Research and development activities associated with the discovery and development of new drugs and products for the treatment of diseases of the eye are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect our research and development expenses to increase as we continue to conduct clinical trials for our product candidates, or if the FDA requires us to conduct additional trials for approval.

Our research and development expenditures are subject to numerous uncertainties in timing and cost to completion. Development timelines, the probability of success and development expenses can differ materially from expectations. The cost of clinical trials may vary significantly over the life of a project as a result of differences arising during clinical development, including, among others, the following:

- number of trials required for approval;
- number of sites included in the trials;
- length of time required to enroll suitable patients;
- number of patients that participate in the trials;
- drop-out or discontinuation rates of patients;
- duration of patient follow-up;
- costs related to compliance with regulatory requirements;
- number and complexity of analyses and tests performed during the trial;
- phase of development of the product candidate; and

efficacy and safety profile of the product candidate.

66

Table of Contents

Our expenses related to clinical trials are based on estimates of patient enrollment and related expenses at clinical investigator sites as well as estimates for the services received and efforts expended pursuant to contracts with research institutions, consultants and CROs that conduct and manage clinical trials on our behalf. We generally accrue expenses related to clinical trials based on contracted amounts applied to the level of patient enrollment and activity according to the protocol. If future timelines or contracts are modified based upon changes in the clinical trial protocol or scope of work to be performed, we modify our estimates of accrued expenses accordingly on a prospective basis. Historically, such modifications have not been material.

As a result of the uncertainties discussed above, we are unable to determine with certainty the duration and completion costs of our development programs or precisely when and to what extent we will receive revenue from the commercialization and sale of our products. We may never succeed in achieving regulatory approval for one or more of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including the uncertainties of future preclinical studies and clinical trials, uncertainties in the clinical trial enrollment rate and changing government regulation. In addition, the probability of success for each product candidate will depend on numerous factors, including efficacy and tolerability profiles, manufacturing capability, competition, and commercial viability.

Other Income (Expense), Net

Other income consists of interest earned on our cash and cash equivalents and investments as well as the net proceeds from the sale of our net operating loss tax benefits for the state of New Jersey. Interest income is not considered significant to our historical financial statements and consists of interest earned on our cash and cash equivalents. Refer to Note 3 to our audited financial statements appearing elsewhere in this report for further information.

Other expense consists of interest expense under our convertible notes, including the 2014 Convertible Notes, amortization of debt discounts and non-cash expense related to changes in the fair value of our warrants liability arising from stock purchase warrants. Prior to our IPO, we recognized all of our outstanding warrants as liabilities on our balance sheet as they were subject to price protection provisions. The liability was revalued at each reporting period and changes in the fair value of the warrant liability were included as a component of Other income (expense), net. Upon closing of the IPO, the remaining outstanding warrants to purchase convertible preferred stock were automatically converted into warrants to purchase common stock and all price protection provisions associated with the warrants were removed, at which time the liabilities were revalued and reclassified to equity. Refer Note 8 and Note 12 to our audited financial statements appearing elsewhere in this report for further information.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of financial condition and results of operations are based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP. The preparation of financial statements also requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, costs and expenses and related disclosures. We evaluate our estimates and judgments on an ongoing basis. Significant estimates include assumptions used in the determination of the fair value measurement of stock purchase warrants, stock-based compensation and certain research and development expenses. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are more fully described in Note 2 to our audited financial statements included elsewhere in this report. The following accounting policies are the most critical in fully understanding and evaluating our reported financial results and affect significant judgments and estimates that we use in the preparation of our financial statements.

Accrued Expenses

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with applicable vendor personnel to

identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued expenses include:

67

Table of Contents

fees paid to CROs in connection with clinical trials;
fees paid to investigative sites in connection with clinical trials; and
fees paid to contract manufacturing organizations and service providers that assist in conducting preclinical studies. We accrue our expenses related to clinical trials based on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct research activities and/or manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the level of effort varies from our estimate, we will adjust the accrual accordingly.

If we underestimate or overestimate the level of services performed or the costs of these services, our actual expenses could differ from our estimates. Although we do not currently anticipate the future settlement of existing accruals to differ materially from our estimates, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low for any period. There have been no material changes in estimates for the periods presented.

Fair Value Measurements

We record certain financial assets and liabilities at fair value based on the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants. The fair value of our financial instruments, including cash and cash equivalents, short-term investments, other current assets, accounts payable and accrued expenses approximate their respective carrying values due to the short-term nature of these instruments. The carrying amounts of long-term investments represent their estimated fair values. The estimated fair value of our 2014 Convertible Notes was \$163.8 million as of December 31, 2014. Refer to Note 5 to our audited financial statements included elsewhere in this report. As of December 31, 2014 and 2013, all outstanding warrants are classified as equity and are recorded within additional paid-in capital on the balance sheet. Refer to Note 12 to our audited financial statements included elsewhere in this report.

Stock-Based Compensation

We recognize compensation costs related to stock options granted to employees ratably over the requisite service period, which in most cases is the vesting period of the award for employees, based on the estimated fair value of the awards on the date of grant. Compensation expense for options granted to non-employees is determined as the fair value of consideration received or the fair value of the equity instruments issued, whichever is more reliably measured. The fair value of the awards granted to non-employees is re-measured each period until the related service is complete. Compensation expense related to restricted stock awards is based on the market value of our common stock on the grant date and is expensed ratably over the vesting period. Compensation expense for stock purchase rights under our employee stock purchase plan is measured and recognized on the date that we become contingently obligated to issue shares of our common stock and is based on the difference between the fair value of our common stock and the purchase price on such date.

Stock-based compensation expense was \$9.2 million, \$2.9 million and \$0.4 million for the years ended December 31, 2014, 2013 and 2012, respectively. As of December 31, 2014, we had \$23.1 million of unrecognized compensation expense.

The intrinsic value of all stock options outstanding as of December 31, 2014 was \$79.8 million, of which \$35.9 million and \$43.9 million related to stock options that were vested and unvested, respectively, at that date.

The intrinsic value of all stock options outstanding as of December 31, 2013 was \$50.4 million, of which \$18.1 million and \$32.3 million related to stock options that were vested and unvested, respectively, at that date.

Table of Contents

Significant Factors, Assumptions and Methodologies Used in Determining Fair Value

Determining the appropriate fair value measurement of stock-based awards requires the use of subjective assumptions. In the absence of a public trading market for our common stock prior to the completion of our IPO, we conducted periodic assessments of the valuation of our common stock. The determination of the fair value measurement of options using the Black-Scholes option pricing model is affected by our estimated common stock fair values as well as assumptions regarding a number of other subjective variables. These other variables include the expected term of the options, our expected stock price volatility over the expected term of the options, stock option exercise and cancellation behaviors, risk-free interest rates and expected dividends.

We estimated the fair value of stock options at the grant date using the following assumptions:

Fair Value of our Common Stock. For all stock options granted after the completion of our IPO, the fair value for our underlying common stock is determined using the closing price on the date of grant as reported on the NASDAQ Global Market. For all stock options granted prior to the completion of our IPO, the fair value for our underlying common stock was determined by our board of directors in its sole discretion based on recommendations from management and taking into account advice and assistance provided by third-party valuation consultants engaged to assist us in connection with such valuations.

Volatility. We calculate expected volatility based on reported data for a selected group of similar publicly traded companies, or guideline peer group, for which the relevant historical information is available. We selected representative companies from the pharmaceutical industry with similar characteristics to us, including stage of product development and therapeutic focus. We will continue to use the guideline peer group volatility information until relevant historical volatility data for our common stock is available to measure expected volatility for future option grants.

Expected Term. We used the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, **Share-Based Payment**, as we do not have sufficient historical exercise and post-vesting termination data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees.

Risk-free Rate. The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities similar to the expected time to exercise.

Forfeiture. Forfeitures are estimated such that we only recognize expense for the shares expected to vest, and adjustments are made if actual forfeitures differ from those estimates. We estimate our annual forfeiture rates based on our historical analysis of actual stock option forfeitures and our future expectations.

Dividend Yield. Except for a one-time cash dividend related to the spin-off of certain non-core intellectual property (further described in Note 15 to our audited financial statements appearing elsewhere in this report), we have never declared or paid any cash dividends and do not presently plan to pay cash dividends in the foreseeable future.

The estimation of the number of stock awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from our current estimates, such amounts will be recorded as a cumulative adjustment in the period in which estimates are revised.

Key assumptions utilized in the fair value calculation for the underlying common stock as of December 31, 2014, 2013 and 2012 appear on the table below.

	YEAR ENDED				
	DECEMBER 31,				
	2014	2013	2012		
Expected term (years)	6.25	6.25	6.25		
Expected stock price volatility ⁽¹⁾	80.44	% 79.20	% 60.00	%	%
Risk-free interest rate	1.90	% 1.78	% 1.05	%	%
Dividend yield	—	—	—		

⁽¹⁾ Weighted Average for 2014

Table of Contents

Stock Purchase Warrants

We account for our stock purchase warrants as either equity or liabilities based upon the characteristics and provisions of the underlying instruments. Warrants classified as equity are recorded as additional paid-in capital on the balance sheet and no further adjustments are made to their valuation. Warrants classified as liabilities are recorded at their fair value on the date of issuance and are re-measured on each subsequent balance sheet date until the earlier of the exercise or expiration of the applicable warrants or until such time that the warrants are no longer determined to be derivative instruments. The fair value changes are recognized as income (decreases in fair value) or expense (increases in fair value) in Other income (expense), net in the statements of operations and comprehensive loss. The fair value of these liabilities is estimated using the Black-Scholes method, which, under our facts and circumstances, approximates, in all material respects, the values determined when using a Monte Carlo simulation.

The Black-Scholes method and the Monte Carlo simulation require the use of subjective assumptions, including but not limited to stock price volatility, the expected life of the warrants, the risk free interest rate and the fair value of the underlying equity securities. The fair value of the underlying common stock is determined as discussed above under “—Stock-Based Compensation.”

Tax Valuation Allowance

A valuation allowance is recorded if it is more likely than not that a deferred tax asset will not be realized. We provided a full valuation allowance on our deferred tax assets that primarily consist of cumulative federal net operating losses of \$133.6 million for the period from inception (June 22, 2005) to December 31, 2014. Due to our three year cumulative loss position, history of operating losses and losses expected to be incurred in the foreseeable future, a full valuation allowance against our net deferred tax assets was considered necessary.

Results of Operations

Comparison of the Years Ended December 31, 2014 and 2013

The following table summarizes the results of our operations for the years ended December 31, 2014 and 2013:

	YEAR ENDED DECEMBER 31, 2014		2013		INCREASE (DECREASE)	% INCREASE (DECREASE)
	(in thousands)					
Expenses						
General and administrative	\$ (20,103)	\$ (10,287)	\$ 9,816	95 %
Research and development	(29,869)	(11,883)	17,986	151 %
Other income (expense), net	1,839		(8,978)	10,817	N/A
Net loss	\$ (48,133)	\$ (31,148)		

General and administrative expenses

General and administrative expenses increased by \$9.8 million for the year ended December 31, 2014 as compared to the year ended December 31, 2013. This increase was primarily associated with the expansion of our employee base to support the growth of our operations. Personnel costs increased by \$6.7 million, including employee stock based compensation expense of \$5.2 million and new salaried employees and related expenses of \$1.9 million. This increase in personnel costs was partially offset by a decrease in severance expense of \$0.4 million related to a former employee. As a result of increased audit fees, legal fees, board expenses and other business related activities in connection with operating as a public company, outside professional fees increased by \$2.8 million and travel expenses increased by \$0.3 million.

Research and development expenses

For the year ended December 31, 2014, our research and development activity was primarily associated with Phase 3 registration trials for Rhopressa™, Phase 2b clinical trials for Roclatan™ and preparatory activities for our Phase 3 clinical trials for Roclatan™. Research and development expenses increased by \$18.0 million for the year ended

December 31, 2014 as compared to the year ended December 31, 2013. Costs for RhopressaTM increased by \$14.8 million as direct clinical costs and

70

Table of Contents

direct non-clinical costs increased \$9.4 million and \$5.4 million, respectively. Costs for Roclatan™ increased \$1.9 million as direct clinical costs increased \$1.6 million and direct non-clinical costs increased \$0.2 million.

Additionally, unallocated expenses including employee compensation, consulting costs and related expenses increased by \$4.9 million. Research and development expenses for product candidates for which further advancement was discontinued decreased by \$3.5 million.

Other income (expense), net

Other income (expense), net increased by \$10.8 million for the year ended December 31, 2014 as compared to the year ended December 31, 2013. The increase was mainly due to a decrease of \$3.7 million in non-cash expense related to the change in the fair value of warrant liabilities and a decrease of \$3.2 million in interest and amortization expense related to our 2014 Convertible Notes and 2012 Convertible Notes. Upon closing of the IPO in October 2013, the principal and accrued interest outstanding under our 2012 Convertible Notes were converted into 1,860,363 shares of common stock resulting in a \$2.7 million loss. Additionally, for the years ended December 31, 2014 and 2013, we received \$2.3 million and \$1.3 million, respectively, of income from the sale of deferred state tax benefits to an unrelated third party, as further described in Note 9 to our audited financial statements appearing elsewhere in this report.

Comparison of the Years Ended December 31, 2013 and 2012

The following table summarizes the results of our operations for the years ended December 31, 2013 and 2012:

	YEAR ENDED DECEMBER 31,		INCREASE	% INCREASE	
	2013	2012	(DECREASE)	(DECREASE)	
	(in thousands)				
General and administrative expenses	\$(10,287) \$(5,020) \$5,267	105	%
Research and development expenses	(11,883) (9,273) \$2,610	28	%
Other income (expense), net	(8,978) (685) \$(8,293) N/A	
Net loss	\$(31,148) \$(14,978)		

General and administrative expenses

General and administrative expenses increased by \$5.3 million for the year ended December 31, 2013 as compared to the year ended December 31, 2012. This increase was primarily associated with an increase of \$3.3 million in personnel costs, including employee stock based compensation and new salaried employees and related expenses resulting in part from the hiring of our Chief Financial Officer in October 2012 and our President and Chief Operating Officer in August 2013. As a result of increased audit fees, legal fees, board expenses and other business related activities in connection with our IPO and operating as a public company, outside professional fees increased by \$1.8 million.

Research and development expenses

For the year ended December 31, 2013, our research and development activity was primarily associated with Phase 2 clinical trials and other non-clinical preparatory activities for Rhopressa™ and product candidates for which further advancement was discontinued during the second quarter of 2013. These product candidates did not demonstrate the efficacy endpoint required for further advancement. Research and development expenses increased by \$2.6 million for the year ended December 31, 2013 as compared to the year ended December 31, 2012. Costs for Rhopressa™ increased by \$1.7 million as direct non-clinical costs and direct clinical costs increased \$1.1 million and \$0.6 million, respectively. Costs for Roclatan™ increased \$0.7 million as direct non-clinical costs increased \$0.5 million and direct clinical costs increased \$0.2 million. Research and development expenses for product candidates for which further advancement was discontinued decreased by \$0.2 million as direct non-clinical costs decreased by \$1.7 million and direct clinical costs increased by \$1.5 million. The remaining change was due to the change in unallocated expenses, including employee salary and related expenses.

Other income (expense), net

Other income (expense), net decreased by \$8.3 million for the year ended December 31, 2013 as compared to the year ended December 31, 2012. The decrease was mainly due to a \$3.7 million increase in non-cash interest expense relating to the amortization of debt discounts and accrued interest, a \$3.2 million unfavorable non-cash change in the fair value of warrant liabilities and a \$2.7 million loss recognized in connection with the conversion of our outstanding convertible notes upon the

71

Table of Contents

closing of our IPO. These increased expenses were partially offset by \$1.3 million of income generated as a result of our participation in the New Jersey Economic Development Authority's sponsored Technology Business Tax Certificate Transfer Program.

Liquidity and Capital Resources

Since our inception, we have funded operations primarily through the sale of equity securities, including our IPO, and the issuance of convertible notes. We have incurred losses and experienced negative operating cash flows since our inception and anticipate that we will continue to incur losses for at least the next several years.

Prior to our IPO, we raised net cash proceeds of \$78.6 million from the private placement of \$43.8 million of convertible preferred stock and \$34.8 million of convertible notes. Subsequent to their issuance, we paid \$0.5 million in cash payments on the convertible notes, \$16.2 million of the convertible notes were converted into shares of convertible preferred stock, which were subsequently converted into shares of common stock in connection with our IPO, and \$18.0 million of the convertible notes to related parties were converted into shares of common stock in connection with our IPO. In connection with our IPO, all outstanding shares of convertible preferred stock were converted into shares of common stock.

On October 30, 2013, we completed our IPO and issued 7,728,000 shares of our common stock at an IPO price of \$10.00 per share, including 1,008,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares to cover over-allotments. We received net proceeds from the IPO of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million.

On September 30, 2014, we issued \$125.0 million aggregate principal amount of the 2014 Convertible Notes. We received net proceeds from the issuance of the 2014 Convertible Notes of approximately \$124.1 million, after deducting discounts and certain expenses of \$875,000.

As of December 31, 2014, our principal sources of liquidity were our cash and cash equivalents and investments, which totaled approximately \$158.2 million.

We believe that our cash and cash equivalents and investments as of December 31, 2014 will provide sufficient resources to complete all currently known non-clinical and clinical requirements for our development programs advancing Rhopressa™ and Roclatan™, and approval by the FDA and product commercialization, pending successful outcome of the trials. Our ability to continue as a going concern will depend, in large part, on our ability to successfully commercialize our product candidates and generate positive cash flow from operations, neither of which is certain.

On November 3, 2014, we filed a shelf registration statement on Form S-3, which was declared effective by the SEC on November 10, 2014. The shelf registration statement permits: (i) the offering, issuance and sale by us of up to a maximum aggregate offering price of \$150.0 million of our common stock; (ii) sales of common stock by certain selling stockholders; and (iii) the offering, issuance and sale of up to a maximum aggregate offering price of \$50.0 million of our common stock that may be issued and sold by us under an "at-the-market" sales agreement with Cantor Fitzgerald & Co. The common stock that may be offered, issued and sold by us under the "at-the-market" sales agreement is included in the \$150.0 million of common stock that may be offered, issued and sold by us under the shelf registration statement. As of December 31, 2014, we have not sold any securities registered pursuant to the shelf registration statement.

The following table summarizes our sources and uses of cash:

(in thousands)	YEAR ENDED		
	DECEMBER 31,		
	2014	2013	2012
Net cash (used in) provided by:			
Operating activities	\$ (33,726) \$ (16,448) \$ (14,968
Investing activities	(73,318) (63) (51
Financing activities	122,981	83,235	2,876

Net increase (decrease) in cash and cash equivalents \$ 15,937 \$66,724 \$(12,143)

During the years ended December 31, 2014, 2013 and 2012, our operating activities used net cash of \$33.7 million, \$16.4 million and \$15.0 million, respectively. The use of net cash in each of these periods primarily resulted from our net losses, adjusted for certain non-cash items. The increase in net loss from operations for the year ended December 31, 2014 as compared to the year ended December 31, 2013 and for the year ended December 31, 2013 as compared to the year ended December 31, 2012 was due primarily to increases in general and administrative expenses and research and development

Table of Contents

expenses as previously described, see “Results of Operations.” For the years ended December 31, 2014 and 2013, we received \$2.3 million and \$1.3 million, respectively, of cash proceeds from the sale of deferred state tax benefits to an unrelated third party, as further described in Note 9 to our audited financial statements appearing elsewhere in this report, which decreased net cash used in operating activities.

During the year ended December 31, 2014, our investing activities used net cash of \$73.3 million primarily related to purchases of available-for-sale investments of \$95.4 million, which was partially offset by maturities and sales of available-for-sale investments of \$20.7 million and \$1.5 million, respectively. During the years ended December 31, 2013 and 2012, our investing activities primarily related to purchases of office furnishings and equipment to facilitate our increased research and development activities and headcount.

During the years ended December 31, 2014, 2013 and 2012, our financing activities provided net cash of \$123.0 million, \$83.2 million and \$2.9 million, respectively. The net cash provided by financing activities during the year ended December 31, 2014 was primarily related to net proceeds of \$124.2 million from the issuance of the 2014 Convertible Notes, partially offset by payments of debt issuance costs of \$1.3 million. The net cash provided by financing activities during the year ended December 31, 2013 was related to net proceeds of \$68.3 million from our IPO and \$15.0 million from the sale of the 2012 Convertible Notes. The net cash provided by financing activities during the year ended December 31, 2012 was related to \$3.0 million from the sale of the 2012 Convertible Notes.

Operating Capital Requirements

We expect to incur increasing operating losses for at least the next several years as we continue to conduct Phase 3 clinical trials for Rhopressa™ and initiate and complete for Phase 3 clinical trials for Roclatan™. We expect that our existing cash and cash equivalents and investments will provide sufficient resources to complete all currently known non-clinical and clinical requirements for our development programs advancing Rhopressa™ and Roclatan™, and approval by the FDA and product commercialization, pending successful outcome of the trials.

We will also continue to incur increasing costs associated with the growth of our operations, including but not limited to, increased costs and expenses for directors fees, increased personnel costs, increased directors and officers insurance premiums, audit and legal fees, investor relations fees, expenses for compliance with reporting requirements under the Exchange Act and rules implemented by the SEC and NASDAQ and various other costs.

Due to the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. We based our projections on assumptions that may prove to be incorrect or unreliable or may change due to circumstances beyond our control, and as a result we may consume our available capital resources earlier than we originally projected. Our future funding requirements will depend on many factors, including, but not limited to the following:

- timing and costs of our future preclinical studies and clinical trials for our product candidates;
- costs of any follow-on development or products;
- timing and cost of the ongoing supportive preclinical studies and activities for our product candidates;
- outcome, timing and costs of seeking regulatory approval;
- costs of commercialization activities for our product candidates, if we receive regulatory approval, including the costs and timing of establishing product sales, marketing, manufacturing and distribution capabilities;
- costs of operating as a public company, including legal, compliance, accounting and investor relations expenses;
- terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish; and
- filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims.

We may need to obtain additional financing to fund our future operations, including supporting sales and marketing activities, as well as funding the ongoing development of any additional product candidates we might license, acquire or develop internally. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity

Table of Contents

securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders.

If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or discontinue our research and development programs or commercialization efforts.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2014:

	TOTAL	LESS THAN 1 YEAR	1 TO 3 YEARS	3 TO 5 YEARS	MORE THAN 5 YEARS
	(in thousands)				
Operating lease obligations ⁽¹⁾	\$2,092	\$460	\$ 641	\$ 724	\$267
Convertible Notes ⁽²⁾	125,000	—	—	—	125,000
	127,092	460	641	724	125,267

(1) Our operating lease obligations are related to our office in New Jersey, research facility in North Carolina and office in Newport Beach, California.

On September 30, 2014, we issued the 2014 Convertible Notes to Deerfield Partners, L.P., Deerfield International Master Fund, L.P., Deerfield Private Design Fund III, L.P., Deerfield Special Situations Fund, L.P. and Deerfield

(2) Special Situations International Master Fund, L.P. On January 1, 2015, Deerfield International Master Fund, L.P. transferred all of its rights under the 2014 Convertible Notes to Deerfield Special Situations Fund, L.P. The 2014 Convertible Notes mature on the seventh anniversary from the date of issuance, unless earlier converted. Refer to Note 8 to our audited financial statements appearing elsewhere in this report for further information.

In January 2015, we entered into a lease agreement (the "Irvine Lease Agreement") under which we are leasing approximately 14,500 square feet of office space in Irvine, California. The initial term of the lease expires in January 2021. Total additional rental payments of approximately \$2.9 million under the Irvine Lease Agreement are expected to be incurred from January 2015 to January 2021 and are excluded from the table above.

We have no other contractual obligations or commitments that are not subject to our existing financial statement accrual processes.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements as defined under SEC rules.

Net Operating Loss Carry-Forwards

We have incurred significant net operating losses since our inception in June 2005. We expect to continue to incur net operating losses for the foreseeable future as we continue to develop our product portfolio, seek regulatory approval, and, if such approval is obtained, prepare to commercialize our products.

As of December 31, 2014, we had approximately \$133.6 million of net operating loss carry-forwards, which may be utilized against future federal income taxes. These net operating losses will begin to expire at various dates beginning in 2024, if not utilized. We also have federal research and development tax credit carry-forwards of \$0.6 million to offset future federal income taxes, which expire at various dates beginning in 2024, if not utilized.

Net operating loss and tax credit carry-forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, as well as similar state provisions.

This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of our company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years.

Table of Contents

As of December 31, 2014, we recorded a full valuation allowance against our net deferred tax assets and development tax credit carry-forwards, as we believe, based on our history of operating losses, it is more likely than not that the tax benefits will not be realized. In the future, if we determine that a portion or all of the tax benefits associated with our tax carry-forwards will be realized, net income would increase in the period of such determination. In January 2015, we participated in the New Jersey Economic Development Authority's Sponsored Technology Business Tax Certificate Transfer Program, as further described in Note 17 to our audited financial statements appearing elsewhere in this report.

Jumpstart Our Business Startups Act of 2012

The Jumpstart Our Business Startups Act of 2012 provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (the "FASB") issued ASU 2014-15, which provides guidance about management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. The new standard is effective for us for the annual period ending after December 15, 2016 and for annual and interim periods thereafter, with early adoption permitted. We are currently evaluating the impact of this accounting standard update on our financial statements.

In June 2014, the FASB issued ASU 2014-10, which eliminates the concept of a development stage entity in its entirety from current accounting guidance. The new standard is effective for us for interim and annual periods beginning after December 15, 2014, with early adoption permitted. We evaluated and elected early adoption of ASU 2014-10 for our annual filing on Form 10-K for the year ended December 31, 2014 and we no longer disclose inception-to-date information in our Statements of Operations and Comprehensive Loss, Cash Flows and Stockholders' Equity (Deficit) and the related notes thereto.

In July 2013, the FASB issued ASU 2013-11, which is an amendment to the accounting guidance on income taxes. This guidance provides clarification on the financial statement presentation of an unrecognized benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. The amendment was effective for us for interim and annual periods beginning after December 15, 2013. The adoption of the provisions of this guidance did not have a material impact on our financial statements.

In February 2013, the FASB issued ASU 2013-02 Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income, which requires that public and non-public companies present information about reclassification adjustments for accumulated other comprehensive income in their annual financial statements in a note or on the face of the financial statements. Public companies are also required to provide this information in interim financial statements. The new disclosure requirements are effective for fiscal years, and interim periods within those years, beginning after December 15, 2012. The adoption of the provisions of this guidance did not have a material impact on our results of operations, cash flows and financial position.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. Our cash and cash equivalents as of December 31, 2014, totaled \$85.6 million and consisted primarily of cash and money market funds with original maturities of three months or less from the date of purchase. Our investments totaled \$72.6 million as of December 31, 2014 and consisted of certificates of deposit, corporate bonds and government agency securities. We had cash and cash equivalents on hand of \$69.6 million as of December 31, 2013. Given the short-term nature of our cash equivalents and investments and our investment policy, a sudden change in market interest rates would not be expected to have a material impact on our financial condition or results of operations. We do not engage in any hedging activities against changes in interest rates. The 2014

Convertible Notes carry a fixed interest rate and, as such, are not subject to interest rate risk. We do not have any foreign currency or other derivative financial instruments.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements required by this item are set forth beginning at page F-1 of this report and are incorporated herein by reference.

75

Table of Contents

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of disclosure controls and procedures.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15(d)-15(e)), as of the end of the period covered by this report. Based upon the evaluation, the Chief Executive Officer and Chief Financial Officer concluded that, as of the end of such period, the disclosure controls and procedures were effective to provide reasonable assurance that information required to be disclosed in the reports we file and submit under the Exchange Act, is (i) recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (ii) accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Management's Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) under the Exchange Act as a process designed by, or under the supervision of, our principal executive officer and principal financial officer and effected by our board of directors, management, and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of our financial statements for external reporting purposes in conformity with generally accepted accounting principles and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in conformity 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 consolidated financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. 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.

Under the supervision of and with the participation of our principal executive officer and principal financial officer, our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2014 based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in 2013 in Internal Control-Integrated Framework. Based on this assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2014.

This annual report on Form 10-K does not include an attestation report of our independent registered public accounting firm regarding internal control over financial reporting. Management's report was not subject to attestation by our independent registered public accounting firm pursuant to an exemption under Section 989G of the

Dodd-Frank Wall Street Reform and Consumer Protection Act made available to us under the Jumpstart Our Business Startups Act of 2012.

Changes in Internal Control Over Financial Reporting

There have been no significant changes in our internal control over financial reporting during our most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

76

Table of Contents

ITEM 9B. OTHER INFORMATION

On November 3, 2014, we entered into an at-the-market sales agreement with Cantor Fitzgerald & Co. In accordance with the terms of the sales agreement, we may offer and sell shares of our common stock having an aggregate offering price of up to \$50.0 million from time to time through Cantor Fitzgerald & Co., acting as agent. The common stock will be issued pursuant to our registration statement on Form S-3 (File No. 333-199821).

Pursuant to the at-the-market sales agreement, shares of common stock may be offered and sold through Cantor Fitzgerald & Co. in transactions that are deemed to be “at-the-market” offerings as defined in Rule 415 of the Securities Act, including sales made directly on or through the NASDAQ Global Market, sales made to or through a market maker other than on an exchange or otherwise, in negotiated transactions at market prices prevailing at the time of sale or at prices related to such prevailing market prices, and/or any other method permitted by law, including in privately negotiated transactions. Cantor Fitzgerald & Co. will act as sales agent on a best efforts basis and use commercially reasonable efforts to sell on our behalf all of the shares of common stock requested to be sold by us, consistent with its normal trading and sales practices, on mutually agreed terms between Cantor Fitzgerald & Co. and us. Except as otherwise described in the at-the-market sales agreement, Cantor Fitzgerald & Co. will be entitled to compensation at a commission rate of up to 3.0% of the gross sales price per share sold. We have no obligation to sell any shares under the at-the-market sales agreement, and may at any time suspend offers under the at-the-market sales agreement or terminate the at-the-market sales agreement.

The summary of the at-the-market sales agreement in this report does not purport to be complete and is qualified by reference to such agreement, which is filed as Exhibit 1.1 to this report.

Table of Contents

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this item is incorporated by reference to the information set forth in the sections titled “Information about Our Board of Directors,” “Information about Our Executive Officers,” “Information about the Board of Directors and Corporate Governance,” “Code of Ethics,” “Section 16(a) Beneficial Ownership Reporting Compliance,” “Committees of the Board of Directors—Nominating and Corporate Governance Committee,” “Committees of the Board of Directors—Audit Committee” and “Report of the Audit Committee of the Board of Directors” in our Proxy Statement.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this item is incorporated by reference to the information set forth in the sections titled “Executive and Director Compensation” and “Committees of the Board of Directors—Compensation Committee” in our Proxy Statement.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The information required by this item is incorporated by reference to the information set forth in the sections titled “Securities Authorized for Issuance under Equity Compensation Plans” and “Security Ownership of Certain Beneficial Owners and Management” in our Proxy Statement.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The information required by this item is incorporated by reference to the information set forth in the sections titled “Board of Directors Independence” and “Transactions with Related Persons” in our Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this item is incorporated by reference to the information set forth in the sections titled “Independent Registered Public Accounting Firm Fees and Services” in our Proxy Statement.

Table of Contents

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

The financial statement schedules and exhibits filed as part of this annual report on Form 10-K are as follows:

(a)(1) Financial Statements

Reference is made to the audited financial statements included in Item 8 of Part II of this report.

(a)(2) Financial Statement Schedules

Financial statement schedules have been omitted because the required information is not present, or not present in amounts sufficient to require submission of the schedules, or because the required information is provided in the financial statements or notes thereto.

(a)(3) Exhibits

The exhibits required to be filed as part of this report are listed in the Exhibit Index attached hereto and are incorporated herein by reference.

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.

AERIE PHARMACEUTICALS, INC.

Date: February 27, 2015

By: /S/ VICENTE ANIDO, JR., PHD
Vicente Anido, Jr., PhD
Chief Executive Officer, Chairman of the Board
(Principal Executive Officer)

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed by the following persons in the capacities and on the dates indicated.

SIGNATURE	TITLE	DATE
/S/ VICENTE ANIDO, JR., PHD Vicente Anido, Jr., PhD	Chief Executive Officer, Chairman of the Board (Principal Executive Officer)	February 27, 2015
/S/ RICHARD J. RUBINO Richard J. Rubino	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 27, 2015
/S/ GERALD D. CAGLE, PHD Gerald D. Cagle, PhD	Director	February 27, 2015
/S/ GEOFFREY DUYK, MD, PHD Geoffrey Duyk, MD, PhD	Director	February 27, 2015
/S/ MURRAY A. GOLDBERG Murray A. Goldberg	Director	February 27, 2015
/S/ DAVID W. GRYSKA David W. Gryska	Director	February 27, 2015
/S/ BENJAMIN F. MCGRAW III, PHARM.D. BENJAMIN F. MCGRAW III, PHARM.D.	Director	February 27, 2015
/S/ ANAND MEHRA, MD Anand Mehra, MD	Director	February 27, 2015

Table of Contents

INDEX TO FINANCIAL STATEMENTS

AERIE PHARMACEUTICALS, INC.

	PAGE
<u>Report of Independent Registered Public Accounting Firm</u>	F-2
<u>Financial Statements</u>	
<u>Balance Sheets</u>	F-3
<u>Statements of Operations and Comprehensive Loss</u>	F-4
<u>Statements of Stockholders' Equity (Deficit)</u>	F-5
<u>Statements of Cash Flows</u>	F-7
<u>Notes to the Financial Statements</u>	F-8

Table of Contents

Report of Independent Registered Public Accounting Firm
To the Board of Directors and Stockholders of
Aerie Pharmaceuticals, Inc.

In our opinion, the accompanying Balance Sheets and the related Statements of Operations and Comprehensive Loss, of Stockholders' Equity (Deficit), and of Cash Flows present fairly, in all material respects, the financial position of Aerie Pharmaceuticals, Inc. at December 31, 2014 and 2013, and the results of their operations and their cash flows for three years then ended in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements 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, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP
Florham Park, New Jersey
February 27, 2015

Table of Contents

AERIE PHARMACEUTICALS, INC.

Balance Sheets

(in thousands, except share and per share data)

	DECEMBER 31,	
	2014	2013
Assets		
Current assets		
Cash and cash equivalents	\$85,586	\$69,649
Short-term investments	54,339	—
Prepaid expenses and other current assets	1,122	618
Total current assets	141,047	70,267
Long-term investments	18,275	—
Furniture, fixtures and equipment, net	240	132
Other assets, net	1,523	59
Total assets	\$161,085	\$70,458
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable and other current liabilities	\$8,336	\$3,482
Interest payable	551	—
Total current liabilities	8,887	3,482
Convertible notes, net of discounts	124,156	—
Total liabilities	133,043	3,482
Commitments and contingencies (Note 14)		
Stockholders' equity		
Preferred stock, \$0.001 par value; 15,000,000 shares authorized as of December 31, 2014 and December 31, 2013; None issued and outstanding	—	—
Common stock, \$0.001 par value; 150,000,000 shares authorized as of December 31, 2014 and December 31, 2013; 24,018,577 and 23,285,549 shares issued and outstanding as of December 31, 2014 and December 31, 2013, respectively	24	23
Additional paid-in capital	171,326	162,021
Accumulated other comprehensive loss	(107) —
Accumulated deficit	(143,201) (95,068
Total stockholders' equity	28,042	66,976
Total liabilities and stockholders' equity	\$161,085	\$70,458
The accompanying notes are an integral part of these financial statements.		

Table of Contents

AERIE PHARMACEUTICALS, INC.

Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	YEAR ENDED		
	DECEMBER 31,		
	2014	2013	2012
Operating expenses			
General and administrative	\$(20,103)	\$(10,287)	\$(5,020)
Research and development	(29,869)	(11,883)	(9,273)
Loss from operations	(49,972)	(22,170)	(14,293)
Other income (expense), net	1,839	(8,978)	(685)
Net loss	\$(48,133)	\$(31,148)	\$(14,978)
Net loss attributable to common stockholders—basic and diluted	\$(48,133)	\$(31,598)	\$(15,643)
Net loss per share attributable to common stockholders—basic and diluted	\$ (2.00)	\$ (6.38)	\$ (16.39)
Weighted average number of common shares outstanding—basic and diluted	24,086,651	4,955,760	954,695
Net loss	\$(48,133)	\$(31,148)	\$(14,978)
Unrealized loss on available-for-sale investments	(107)	—	—
Comprehensive loss	\$(48,240)	\$(31,148)	\$(14,978)

The accompanying notes are an integral part of these financial statements.

Table of Contents

AERIE PHARMACEUTICALS, INC.

Statements of Stockholders' Equity (Deficit)

(in thousands, except share and per share data)

	CONVERTIBLE PREFERRED STOCK SHARES	AMOUNT	COMMON STOCK SHARES	AMOUNT	ADDITIONAL PAID-IN CAPITAL	ACCUMULATED OTHER COMPREHENSIVE LOSS	ACCUMULATED DEFICIT	TOTAL
Balances at December 31, 2011	12,120,531	\$ 60,348	951,973	\$ 1	\$ 109	\$ —	\$ (48,807)	\$ (48,697)
Exercise of stock options	—	—	12,907	—	6	—	—	6
Stock-based compensation	—	—	—	—	430	—	—	430
Accretion from conversion of note payable to related parties	—	292	—	—	(290)	—	(2)	(292)
Accretion of stock issuance costs	—	258	—	—	(255)	—	(3)	(258)
Cash dividend	—	—	—	—	—	—	(130)	(130)
Net loss	—	—	—	—	—	—	(14,978)	(14,978)
Balances at December 31, 2012	12,120,531	60,898	964,880	1	—	—	(63,920)	(63,919)
Exercise of stock options	—	—	3,195	—	1	—	—	1
Vesting of restricted stock	—	—	124,966	—	—	—	—	—
Stock-based compensation	—	—	—	—	2,858	—	—	2,858
Accretion from conversion of note payable to related parties	—	240	—	—	(240)	—	—	(240)
Accretion of stock issuance costs	—	210	—	—	(210)	—	—	(210)
Conversion of convertible preferred stock to common stock	(12,120,531)	(61,348)	12,120,531	12	61,336	—	—	61,348
Conversion of convertible notes payable and accrued interest to common stock	—	—	1,860,363	2	18,602	—	—	18,604
	—	—	7,728,000	8	68,218	—	—	68,226

Proceeds from issuance of common stock in initial public offering, net of underwriting discounts and commissions of \$5,410 and offering costs of \$3,644								
Issuance of common stock upon net exercise of warrants	—	—	483,614	—	4,896	—	—	4,896
Reclassification of warrants from liabilities to equity	—	—	—	—	6,560	—	—	6,560
Net loss	—	—	—	—	—	—	(31,148)	(31,148)
Balances at December 31, 2013	—	—	23,285,549	23	162,021	—	(95,068)	66,976
Issuance of common stock upon exercise of stock purchase rights	—	—	10,941	—	119	—	—	119
Exercise of stock options	—	—	579,083	1	8	—	—	9
Vesting of restricted stock	—	—	143,004	—	—	—	—	—
Stock-based compensation	—	—	—	—	9,178	—	—	9,178
Unrealized loss on available-for-sale investments	—	—	—	—	—	(107)	—	(107)
Net loss	—	—	—	—	—	—	(48,133)	(48,133)
Balances at December 31, 2014	—	\$—	24,018,577	\$24	\$ 171,326	\$ (107)	\$ (143,201)	\$28,042

The accompanying notes are an integral part of these financial statements.

Table of Contents

AERIE PHARMACEUTICALS, INC.

Statements of Cash Flows

(in thousands, except share and per share data)

	YEAR ENDED		
	DECEMBER 31,		
	2014	2013	2012
Cash flows from operating activities			
Net loss	\$(48,133)	\$(31,148)	\$(14,978)
Adjustments to reconcile net loss to net cash used in operating activities			
Depreciation	73	64	138
Amortization and accretion costs related to notes payable—related parties	—	3,207	59
Amortization of deferred financing costs and debt discount	77	—	—
Amortization of discount on available-for-sale investments	416	—	—
Interest payable—related parties	—	588	16
Loss (gain) on conversion of notes payable	—	2,737	—
Stock-based compensation	9,178	2,858	430
Change in fair value measurements	—	3,717	630
Changes in operating assets and liabilities			
Prepaid, current and other assets	(717)	(516)	9
Accounts payable and other current liabilities	4,829	2,045	(1,272)
Interest payable	551	—	—
Net cash used in operating activities	(33,726)	(16,448)	(14,968)
Cash flows from investing activities			
Purchase of available-for-sale investments	(95,376)	—	—
Maturity of available-for-sale investments	20,739	—	—
Sale of available-for-sale investments	1,500	—	—
Purchase of furniture, fixtures and equipment	(181)	(63)	(51)
Net cash used in investing activities	(73,318)	(63)	(51)
Cash flows from financing activities			
Proceeds from issuance of convertible notes, net of discounts	124,150	—	—
Payments of debt issuance costs	(1,297)	—	—
Proceeds from issuance of common stock in initial public offering, net of underwriting discounts	—	71,870	—
Payments of initial public offering costs	—	(3,644)	—
Proceeds from notes payable to related parties	—	15,000	3,000
Dividends paid	—	—	(130)
Proceeds from exercise of stock options	9	1	6
Proceeds from exercise of warrants	—	8	—
Proceeds from exercise of stock purchase rights	119	—	—
Net cash provided by financing activities	122,981	83,235	2,876
Net change in cash and cash equivalents	15,937	66,724	(12,143)
Cash and cash equivalents			
Beginning of period	69,649	2,925	15,068
End of period	\$85,586	\$69,649	\$2,925
Supplemental disclosures			
Interest paid	\$—	\$—	\$—
Non-cash financing activities			

Edgar Filing: AERIE PHARMACEUTICALS INC - Form 10-K

Conversion of preferred stock to common stock	\$—	\$61,348	\$—
Conversion of convertible notes payable and accrued interest to common stock	—	18,604	—
Issuance of common stock upon net exercise of warrants	—	4,888	—
Reclassification of warrants from liabilities to equity	—	6,560	—
Debt discount attributable to warrants	—	—	728
Accretion from conversion of note payable to related parties	—	240	292
Accretion of stock issuance costs	—	210	258
Deferred costs from issuance of convertible notes	25	—	—

The accompanying notes are an integral part of these financial statements.

F-6

Table of Contents

AERIE PHARMACEUTICALS, INC.

Notes to the Financial Statements

1. The Company

Aerie Pharmaceuticals, Inc. (the “Company”) is a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of topical, small molecule drugs to treat patients with glaucoma and other diseases of the eye. Incorporated in the State of Delaware on June 22, 2005, the Company has its principal executive offices in Irvine, California. Clinical and finance operations are located in Bedminster, New Jersey and the Company conducts research in Research Triangle Park, North Carolina. Refer to Note 17 for further information regarding our Irvine, California office.

The Company has not yet commenced primary operations or generated product revenue. The Company’s activities since inception primarily consisted of developing product candidates, raising capital and performing research and development activities. The Company has no current source of revenue to sustain its present activities, and it does not expect to generate revenue until and unless it receives regulatory approval of and successfully commercializes its product candidates. The Company has incurred losses and experienced negative operating cash flows since inception. The Company has funded its operations primarily through the sale of equity securities and issuance of convertible notes. In October 2013, the Company completed its initial public offering (“IPO”) and issued 7,728,000 shares of its common stock at an IPO price of \$10.00 per share, including 1,008,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares to cover over-allotments. The Company received net proceeds from the IPO of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million. On September 30, 2014, the Company issued \$125.0 million aggregate principal amount of senior secured convertible notes (the “2014 Convertible Notes”). The Company received net proceeds from the issuance of the 2014 Convertible Notes of approximately \$124.1 million, after deducting discounts and certain expenses of \$875,000. Refer to Note 8 for further information regarding the Convertible Notes.

On November 3, 2014, the Company filed a shelf registration statement on Form S-3, which was declared effective by the SEC on November 10, 2014. The shelf registration statement permits: (i) the offering, issuance and sale of up to a maximum aggregate offering price of \$150.0 million of the Company’s common stock; (ii) sales of common stock by certain selling stockholders; and (iii) the offering, issuance and sale by the Company of up to a maximum aggregate offering price of \$50.0 million of the Company’s common stock that may be issued and sold under an “at-the-market” sales agreement with Cantor Fitzgerald & Co. The common stock that may be offered, issued and sold by the Company under the “at-the-market” sales agreement is included in the \$150.0 million of common stock that may be offered, issued and sold by us under the shelf registration statement. As of December 31, 2014, the Company has not sold any securities registered pursuant to the shelf registration statement.

If the Company does not successfully commercialize any of its product candidates, it may be unable to generate product revenue or achieve profitability. Accordingly, the Company may be required to obtain further funding through other public or private offerings, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to the Company on acceptable terms, or at all. If the Company is unable to raise capital when needed or on attractive terms, it would be forced to delay, reduce or eliminate its research and development programs or commercialization efforts.

2. Significant Accounting Policies

Basis of Presentation

The Company’s financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (“U.S. GAAP”).

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities

at the date of the financial statements and reported amounts of income and expenses during the reporting periods. Significant items subject to such estimates and assumptions include the valuation of stock options and warrants and operating expense accruals. Actual results could differ from those estimates.

F-7

Table of Contents

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment. All operations are located in the United States.

Reverse Stock Split

The Company effected a 1-for-5 reverse stock split on October 8, 2013. Accordingly, all share and per share amounts for all preceding periods presented in these financial statements and notes thereto, have been adjusted retroactively to reflect this reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

Cash Equivalents

Cash equivalents consist of short-term, highly liquid investments with an original term of three months or less at the date of purchase. Cash deposits are in six financial institutions in the United States.

Concentration of Credit Risk

The Company's cash and cash equivalent balances with financial institutions exceed the \$250,000 amount insured by the Federal Deposit Insurance Corporation.

Deferred Financing Costs

Deferred financing costs consist of financing costs incurred by the Company in connection with the closing of the Company's 2014 Convertible Notes and are included in Other assets. The Company amortizes deferred financing costs through the earlier of maturity or the conversion of the 2014 Convertible Notes using the effective interest method. Refer to Note 8 for further information regarding the 2014 Convertible Notes.

Debt Discounts

Debt discounts consist of fees and expenses incurred by the Company in connection with the closing of the Company's 2014 Convertible Notes that were paid directly to the note holders. The Company amortizes debt discounts through the earlier of maturity or the conversion of the 2014 Convertible Notes using the effective interest method. Refer to Note 8 for further information regarding the 2014 Convertible Notes.

Furniture, Fixtures and Equipment, Net

Furniture, fixtures and equipment is recorded at historical cost. Depreciation is calculated using the straight-line method over the estimated useful lives of the related assets. Repairs and maintenance are expensed when incurred. Upon retirement or sale, the cost of the assets disposed of and the related accumulated depreciation are removed from the accounts, and any resulting gain or loss is included in the determination of net income.

Research and Development Costs

Research and development costs are charged to expense as incurred and include, but are not limited to:

- Employee-related expenses including salaries, benefits, travel and stock-based compensation expense for research and development personnel;
- expenses incurred under agreements with contract research organizations, contract manufacturing organizations and service providers that assist in conducting clinical and preclinical studies;
- costs associated with preclinical activities and development activities;
- costs associated with regulatory operations; and
- depreciation expense for assets used in research and development activities.

Costs for certain development activities, such as clinical studies, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to the Company by its vendors on their actual costs incurred. Payments for these activities are based on the terms of the individual

Table of Contents

arrangements, which may differ from the patterns of costs incurred, and are reflected in the financial statements as prepaid or accrued expense, which are reported in accounts payable and other current liabilities (Note 7). No material adjustments to these estimates have been recorded in these financial statements.

Stock-Based Compensation

Compensation cost of stock-based awards granted to employees is measured at grant date, based on the estimated fair value of the award. The Company estimates the fair value of stock options using a Black-Scholes option pricing model. Compensation expense for options granted to non-employees is determined as the fair value of consideration received or the fair value of the equity instruments issued, whichever is more reliably measured. The fair value of restricted stock awards is determined based on the fair value of the Company's common stock on the date of grant. Stock-based compensation costs are expensed on a straight-line basis (net of estimated forfeitures) over the relevant vesting period. The fair value of unvested awards granted to non-employees is re-measured each period until the related service is complete. Compensation expense for employee stock purchase plan rights ("stock purchase rights") is measured and recognized on the date that the Company becomes obligated to issue shares of common stock and is based on the difference between the fair value of the Company's common stock and the purchase price on such date. All stock-based compensation costs are recorded between general and administrative and research and development costs in the statements of operations based upon the underlying employees roles within the Company. No related tax benefits of the stock-based compensation have been recognized.

Investments

The Company determines the appropriate classification of its investments in debt and equity securities at the time of purchase. The Company's investments are comprised of certificates of deposit, commercial paper, corporate bonds and government agency securities that are classified as available-for-sale in accordance with ASC 320, Investments—Debt and Equity Securities. The Company classifies investments available to fund current operations as current assets on its balance sheet. Investments are classified as long-term assets on the balance sheet if (i) the Company has the intent and ability to hold the investments for a period of at least one year and (ii) the contractual maturity date of the investments is greater than one year.

Available-for-sale investments are recorded at fair value, with unrealized gains or losses included in Accumulated other comprehensive gain (loss) on the Company's balance sheets. Realized gains and losses are determined using the specific identification method and are included as a component of Other income (expense), net (Note 3). There were no realized gains or losses recognized for the years ended December 31, 2014, 2013 or 2012.

The Company reviews investments for other-than-temporary impairment whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. To determine whether an impairment is other-than-temporary, the Company considers its intent to sell, or whether it is more likely than not that the Company will be required to sell the investment before recovery of the investment's amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, the severity and the duration of the impairment and changes in value subsequent to period end. As of December 31, 2014, there were no investments with a fair value that was significantly lower than the amortized cost basis or any investments that had been in an unrealized loss position for a significant period.

Fair Value Measurements

The Company records certain financial assets and liabilities at fair value based on the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants. The fair value of the Company's financial instruments, including cash and cash equivalents, short-term investments, other current assets, accounts payable and accrued expenses approximate their respective carrying values due to the short-term nature of these instruments. The carrying amounts of long-term investments represent their estimated fair values. The estimated fair value of the Company's 2014 Convertible Notes was \$163.8 million as of December 31, 2014 (Note 5). As of December 31, 2014 and 2013, all outstanding warrants are classified as equity and are recorded within additional paid-in capital on the balance sheet (Note 12).

Stock Purchase Warrants

The Company accounts for its stock purchase warrants as either equity or liabilities based upon the characteristics and provisions of the underlying instruments. Warrants classified as equity are recorded as additional paid-in capital on the balance sheet and no further adjustments are made to their valuation. Warrants classified as liabilities are recorded at their fair value on the date of issuance and are re-measured on each subsequent balance sheet date until the earlier of the exercise or expiration of the applicable warrants or until such time that the warrants are no longer determined to be derivative instruments. The fair

F-9

Table of Contents

value changes are recognized as income (decreases in fair value) or expense (increases in fair value) in Other income (expense), net in the statements of operations and comprehensive loss. The fair value of these liabilities is estimated using the Black-Scholes method, which, under the Company's facts and circumstances, approximates, in all material respects, the values determined when using a Monte Carlo simulation.

Comprehensive Loss

Comprehensive loss is comprised of net loss and other comprehensive loss. Other comprehensive loss includes changes in stockholders' equity that are excluded from net income (loss), specifically changes in unrealized gains and losses on the Company's available-for-sale securities.

Income Taxes

Deferred tax assets or liabilities are recorded for temporary differences between financial statement and tax basis of assets and liabilities, using enacted rates in effect for the year in which the differences are expected to reverse.

Tax Valuation Allowance

A valuation allowance is recorded if it is more likely than not that a deferred tax asset will not be realized. Due to the Company's three year cumulative loss position, history of operating losses and losses expected to be incurred in the foreseeable future, a full valuation allowance was considered necessary. The Company has provided a full valuation allowance on its deferred tax assets that primarily consist of cumulative federal net operating losses of \$133.6 million for the period from inception (June 22, 2005) to December 31, 2014 (Note 9).

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (the "FASB") issued ASU 2014-15, which provides guidance about management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. The new standard is effective for the Company for the annual period ending after December 15, 2016 and for annual and interim periods thereafter, with early adoption permitted. The Company is currently evaluating the impact of this accounting standard update on the Company's financial statements.

In June 2014, the FASB issued ASU 2014-10, which eliminates the concept of a development stage entity in its entirety from current accounting guidance. The new standard is effective for the Company for interim and annual periods beginning after December 15, 2014, with early adoption permitted. The Company evaluated and elected early adoption of ASU 2014-10 for the year ended December 31, 2014 and the Company no longer discloses inception-to-date information in its Statements of Operations and Comprehensive Loss, Cash Flows and Stockholders' Equity (Deficit) and the related notes thereto.

In July 2013, the FASB issued ASU 2013-11, which is an amendment to the accounting guidance on income taxes. This guidance provides clarification on the financial statement presentation of an unrecognized benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. The amendment is effective for the Company for interim and annual periods beginning after December 15, 2013. The adoption of the provisions of this guidance did not have a material impact on the Company's financial statements.

In February 2013, the FASB issued ASU 2013-02 Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income, which requires that public and non-public companies present information about reclassification adjustments for accumulated other comprehensive income in their annual financial statements in a note or on the face of the financial statements. Public companies are also required to provide this information in interim financial statements. The new disclosure requirements are effective for fiscal years, and interim periods within those years, beginning after December 15, 2012. The adoption of the provisions of this guidance did not have a material impact on the Company's results of operations, cash flows and financial position.

Net Loss per Share Attributable to Common Stock

Basic net loss per share attributable to common stock ("Basic EPS") is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period, without consideration for potentially dilutive securities with the exception of warrants for common stock with a \$0.05 exercise, which are exercisable for nominal consideration, and shares that are unequivocally issuable under the

Company's employee stock purchase plan and are therefore included in the calculation of the weighted-average number of shares of common stock as common stock equivalents. Net loss attributable to common stockholders is calculated by adjusting the Company's net loss for accretion on convertible preferred stock, if any. Diluted net loss per share attributable to common stock ("Diluted EPS") gives

F-10

Table of Contents

effect to all dilutive potential shares of common stock outstanding during this period. For Diluted EPS, net loss attributable to common stockholders used in calculating Basic EPS is adjusted for certain items related to the dilutive securities.

For all periods presented, the Company's potential common stock equivalents have been excluded from the computation of Diluted EPS as their inclusion would have the effect of reducing the net loss per share of common stock. Therefore, the denominator used to calculate Basic EPS and Diluted EPS is the same in all periods presented. The Company's potential common stock equivalents that have been excluded from the computation of Diluted EPS for all periods presented consist of the following:

	DECEMBER 31,		
	2014	2013	2012
2014 convertible notes ⁽¹⁾	5,040,323	—	—
Convertible preferred stock ⁽²⁾	—	—	12,120,531
Outstanding stock options	3,826,459	3,189,660	1,554,200
Notes and interest payable to related parties ⁽²⁾	\$—	\$—	\$3,016,000
Stock purchase warrants	309,506	309,506	595,869
Restricted common stock awards	103,064	246,068	—

Conversion is limited to a 9.985% ownership cap in shares of common stock by the holder. Additionally, the 2014 (1) Convertible Notes provide for an increase in the conversion rate if conversion is elected in connection with a significant corporate transaction. Refer to Note 8 for further information regarding the 2014 Convertible Notes.

In connection with the completion of the Company's IPO, the outstanding shares of convertible preferred stock and (2) outstanding notes to related parties and accrued interest thereon were converted into 12,120,531 and 1,860,363 shares of common stock, respectively.

3. Other Income (Expense), Net

Other income (expense), net consist of the following:

(in thousands)	YEAR ENDED DECEMBER 31,		
	2014	2013	2012
Interest and amortization expense	\$(628)	\$(3,795)	\$(75)
Loss on conversion of notes payable to related parties	—	(2,737)	—
Sale of New Jersey state tax benefit	2,288	1,268	—
Expense due to change in fair value measurements ⁽¹⁾	—	(3,717)	(630)
Other income, net	179	3	20
	\$1,839	\$(8,978)	\$(685)

⁽¹⁾ Includes change in fair value of warrant liabilities and change in fair value of a certain conversion feature related to the 2012 Notes (as defined herein) that was determined to be an embedded derivative requiring bifurcation and separate accounting. See Note 12 and Note 8, respectively.

Table of Contents

4. Investments

Cash, cash equivalents and investments as of December 31, 2014 included the following:

(in thousands)	AMORTIZED COST	GROSS UNREALIZED GAINS	GROSS UNREALIZED LOSSES	FAIR VALUE
Cash and cash equivalents:				
Cash and money market accounts	\$ 84,613	\$ —	\$ —	\$ 84,613
Certificates of deposit	472	—	—	472
Corporate bonds	501	—	—	501
Total cash and cash equivalents	\$ 85,586	\$ —	\$ —	\$ 85,586
Investments:				
Certificates of deposit (due within 1 year)	\$ 25,823	\$ —	\$ (9)	\$ 25,814
Certificates of deposit (due within 2 years)	4,429	1	(3)	4,427
Commercial paper (due within 1 year)	5,988	1	(3)	5,986
Corporate bonds (due within 1 year)	16,487	—	(24)	16,463
Corporate bonds (due within 2 years)	13,912	—	(64)	13,848
Government agencies (due within 1 year)	6,082	—	(6)	6,076
Total investments	\$ 72,721	\$ 2	\$ (109)	\$ 72,614
Total cash, cash equivalents, and investments	\$ 158,307	\$ 2	\$ (109)	\$ 158,200

The Company held only cash and cash equivalents at December 31, 2013 and did not hold any investments.

5. Fair Value Measurements

The Company records certain financial assets and liabilities at fair value in accordance with the provisions of ASC Topic 820 on fair value measurements. As defined in the guidance, fair value, defined as an exit price, represents the amount that would be received to sell an asset or pay to transfer a liability in an orderly transaction between market participants. As a result, fair value is a market-based approach that should be determined based on assumptions that market participants would use in pricing an asset or a liability. As a basis for considering these assumptions, the guidance defines a three-tier value hierarchy that prioritizes the inputs used in the valuation methodologies in measuring fair value.

Level 1—Unadjusted quoted prices in active, accessible markets for identical assets or liabilities.

Level 2—Other inputs that are directly or indirectly observable in the marketplace.

Level 3—Unobservable inputs that are supported by little or no market activity.

The fair value hierarchy also requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value.

Table of Contents

The following tables summarize the fair value of financial assets and liabilities that are measured at fair value and the classification by level of input within the fair value hierarchy:

(in thousands)	FAIR VALUE MEASUREMENTS AS OF DECEMBER 31, 2014			
	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
Cash and cash equivalents:				
Cash and money market accounts	\$84,613	\$—	\$—	\$84,613
Certificates of deposit	—	472	—	472
Corporate bonds	—	501	—	501
Total cash and cash equivalents:	\$84,613	\$973	\$—	\$85,586
Investments:				
Certificates of deposit	\$—	\$30,241	\$—	\$30,241
Commercial paper	—	5,986	—	5,986
Corporate bonds	—	30,311	—	30,311
Government agencies	—	6,076	—	6,076
Total investments	\$—	\$72,614	\$—	\$72,614
Total cash, cash equivalents, and investments:	\$84,613	\$73,587	\$—	\$158,200

(in thousands)	FAIR VALUE MEASUREMENTS AS OF DECEMBER 31, 2013			
	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
Cash and cash equivalents:				
Cash and money market accounts	\$69,649	\$—	\$—	\$69,649
Total cash and cash equivalents:	\$69,649	\$—	\$—	\$69,649

As of December 31, 2014, the estimated fair value of the Company's 2014 Convertible Notes was \$163.8 million. The estimated fair value of the 2014 Convertible Notes was determined using a scenario analysis and Monte Carlo simulation model to capture the various features of the 2014 Convertible Notes. The scenario analysis and Monte Carlo simulation require the use of Level 3 unobservable inputs and subjective assumptions, including but not limited to the probability of conversion, stock price volatility, the risk free interest rate and credit spread. The estimates presented are not necessarily indicative of amounts that could be realized in a current market exchange. The use of alternative market assumptions and estimation methodologies could have a material effect on these estimates of fair value.

The Company had no long-term debt as of December 31, 2013.

6. Furniture, Fixtures and Equipment, Net

Furniture, fixtures and equipment, net consist of the following:

(in thousands)	ESTIMATED USEFUL LIVES (YEARS)	DECEMBER 31,	
		2014	2013
Laboratory equipment	7	\$830	\$789
Software and computer equipment	3	195	142
Furniture and fixtures	5	238	151
		1,263	1,082
Less: Accumulated depreciation		(1,023) (950
		\$240	\$132

Depreciation expense was \$73,000, \$64,000 and \$138,000 for years ended December 31, 2014, 2013 and 2012, respectively.

F-13

Table of Contents

7. Accounts Payable & Other Current Liabilities

Accounts payable and other current liabilities consist of the following:

(in thousands)	DECEMBER 31,	
	2014	2013
Accounts payable	\$2,068	\$1,442
Accrued expenses and other liabilities:		
Employee benefits and compensation related accruals ⁽¹⁾	2,257	966
General and administrative related accruals	731	411
Research and development related accruals	3,280	663
	\$8,336	\$3,482

(1) Comprised of accrued bonus, accrued vacation, accrued severance liabilities, and liabilities under the Company's employee stock purchase plan.

8. Convertible Notes

On September 30, 2014, the Company issued the 2014 Convertible Notes to Deerfield Partners, L.P., Deerfield International Master Fund, L.P., Deerfield Private Design Fund III, L.P., Deerfield Special Situations Fund, L.P. and Deerfield Special Situations International Master Fund, L.P. (collectively, "Deerfield").

The 2014 Convertible Notes bear interest at a rate of 1.75% per annum payable quarterly in arrears on the first business day of each January, April, July and October, commencing on January 1, 2015. The 2014 Convertible Notes mature on the seventh anniversary from the date of issuance, unless earlier converted.

The 2014 Convertible Notes constitute a senior secured obligation of the Company, collateralized by a first priority security interest in substantially all of the assets of the Company. The 2014 Convertible Notes provide that, upon the request of the Company, Deerfield will release all of the liens on the collateral if both of the following occur: (i) beginning one month after U.S. Food and Drug Administration approval of either RhopressaTM or RoclatanTM, shares of the Company's common stock have traded at a price above \$30 per share (subject to adjustment for any subdivision or combination of outstanding common stock) for 30 consecutive trading days, and (ii) the Company is prepared to close a financing that will be secured by a lien on the Company's assets, subject only to the release of the lien on the Company's assets by Deerfield.

At closing, the Company paid Deerfield a one-time transaction fee of \$625,000. In addition, the Company reimbursed Deerfield in the amount of \$250,000 for certain expenses incurred by Deerfield in connection with the transaction.

The Company also incurred \$1.3 million of legal and advisory fees in connection with the transaction.

The 2014 Convertible Notes are convertible at any time at the option of Deerfield, in whole or in part, into shares of common stock, including upon the repayment of the 2014 Convertible Notes at maturity (the "Conversion Option"). However, upon conversion, Deerfield (together with their affiliates) is limited to a 9.985% ownership cap in shares of common stock (the "9.985% Cap"). The 9.985% Cap would remain in place upon any assignment of the 2014 Convertible Notes by Deerfield.

The initial conversion price is \$24.80 per share of common stock (equivalent to an initial conversion rate of 40.32 shares of common stock per \$1,000 principal amount of 2014 Convertible Notes), representing a 30% premium over the closing price of the common stock on September 8, 2014. The conversion rate and the corresponding conversion price are subject to adjustment for stock dividends (other than a dividend for which Deerfield would be entitled to participate on an as-converted basis), stock splits, reverse stock splits and reclassifications. In addition, in connection with certain significant corporate transactions, Deerfield, at its option, may (i) require the Company to prepay all or a portion of the principal amount of the 2014 Convertible Notes, plus accrued and unpaid interest, or (ii) convert all or a portion of the principal amount of the 2014 Convertible Notes into, depending upon the type of transaction, shares of common stock or the right to receive upon consummation of the transaction the consideration Deerfield would have received had Deerfield converted the 2014 Convertible Notes immediately prior to the consummation of the

transaction. The 2014 Convertible Notes provide for an increase in the conversion rate if Deerfield elects to convert their 2014 Convertible Notes in connection with a significant corporate transaction. The maximum increase to the initial conversion rate, in connection with a significant corporate transaction, is 12.07 shares of common stock per \$1,000 principal amount of 2014 Conversion Notes, which decreases over time and is determined by reference to the price of the common stock prior to the consummation of the significant corporate transaction or the value of the significant corporate transaction.

F-14

Table of Contents

The agreement governing the 2014 Convertible Notes contains various representations and warranties, and affirmative and negative covenants, customary for financings of this type, including restrictions on the incurrence of additional debt and liens on the Company's assets. As of December 31, 2014, the Company was in compliance with these covenants. The agreement governing the 2014 Convertible Notes also provides for certain events of default, including the failure to pay principal and interest when due; inaccuracies in the Company's representations and warranties to Deerfield; failure to comply with any of the covenants; the Company's insolvency or the occurrence of certain bankruptcy-related events; certain judgments against the Company; the suspension, cancellation or revocation of governmental authorizations that are reasonably expected to have a material adverse effect on the Company's business; the acceleration of a specified amount of indebtedness; and the failure to deliver shares of common stock upon conversion of the 2014 Convertible Notes. If any event of default were to occur, and continue beyond any applicable cure period, the holders of more than 50% of the aggregate principal amount of the then outstanding 2014 Convertible Notes would be permitted to declare the principal and accrued and unpaid interest to be immediately due and payable.

The Company recorded the 2014 Convertible Notes as long-term debt at face value less debt discounts relating to fees and certain expenses paid to Deerfield in connection with the transaction. The Conversion Option is a derivative that qualifies for an exemption from bifurcation and liability accounting as provided for in ASC Topic 815 "Derivatives and Hedging – Contracts in Entity's Own Equity" ("ASC 815"). Since the Conversion Option is not bifurcated as a derivative pursuant to ASC 815, the Company further evaluated the Conversion Option to determine whether it is considered a beneficial conversion feature ("BCF"). The Company determined that the initial accounting conversion price was greater than the fair value of the common stock at the close of trading on the date of issuance, therefore no BCF existed at inception. However, if Deerfield elects to convert their 2014 Convertible Notes in connection with a significant corporate transaction, the increase to the initial conversion rate may cause a contingent BCF to exist at the time of conversion. The contingent BCF, if any, will be recognized in earnings when the contingency is resolved and will be measured using the fair value of the common stock at the close of trading on the date of issuance and the accounting conversion price as adjusted for such an increase to the initial conversion rate.

As of September 30, 2014, the Company recognized unamortized debt discounts of \$875,000. Debt discounts are amortized using the effective interest method through the earlier of maturity or the conversion of the 2014 Convertible Notes.

The table below summarizes the carrying value of the 2014 Convertible Notes as of December 31, 2014:

(in thousands)	DECEMBER 31, 2014
Gross proceeds	\$ 125,000
Initial value of issuance costs recorded as debt discount	(875)
Amortization of debt discount	31
Carrying value	\$ 124,156

For the year ended December 31, 2014, interest expense related to the 2014 Convertible Notes was \$551,000.

On December 7, 2012, the Company authorized the sale of convertible notes (the "2012 Notes") to related parties in the aggregate principal amount of \$15.0 million. The 2012 Notes accrued interest at a rate of 8% per annum, with principal plus accrued interest thereon due upon maturity at September 30, 2013. The initial closing comprised of five individual convertible notes with an aggregate principal balance of \$3.0 million. As of December 31, 2012, \$12.0 million of 2012 Notes were authorized and available for sale. On March 28, 2013, May 23, 2013 and August 9, 2013, the Company completed the second, third and fourth closing of the 2012 Notes, respectively. The closings each comprised of five individual convertible notes with aggregate principal balances of \$3.0 million, \$4.5 million, and \$4.5 million, respectively. On August 9, 2013, the Company amended the agreements relating to the 2012 Notes. The amendment authorized the sale of an additional \$3.0 million of the 2012 Notes, resulting in an aggregate principal amount of \$18.0 million being authorized. In addition, the amendment extended the maturity date of the 2012 Notes from September 30, 2013 to December 31, 2013 and the issuance period through November 30, 2013. No other terms and conditions of the agreements were changed as part of the amendment. In accordance with ASC 470 Debt, the

amendment met the criteria of a troubled debt restructuring and the amortization of the debt discount was revised to align with a new effective interest rate determined as of the amendment date. No gain was recorded as part of the restructuring. On September 30, 2013, the Company completed the fifth closing of the 2012 Notes. Aggregate proceeds to the Company were \$3.0 million.

On October 30, 2013, upon closing of the IPO, the principal and accrued interest outstanding under the 2012 Notes were converted into 1,860,363 shares of common stock at a conversion price equal to the IPO price of \$10.00 per share. The Company accounted for the conversion as an extinguishment of debt.

F-15

Table of Contents

In connection with the issuance of the 2012 Notes, the Company determined that a certain conversion feature was an embedded derivative requiring bifurcation and separate accounting. To estimate the fair value, the Company compared the net present value of expected cash flows of the issued 2012 Notes with and without the conversion feature comprising the embedded derivative. The Company determined that the fair value of the embedded derivative was immaterial as of August 9, 2013, May 23, 2013, March 28, 2013 and December 7, 2012, representing the fourth, third, second and initial closing dates, and as of December 31, 2012. As of September 30, 2013, the fair value of the embedded derivative was \$96,000. The Company determined that upon the closing of the IPO on October 30, 2013, at which time the 2012 Notes were converted into common stock, no embedded derivative existed. The Company recorded the change in fair value as a component of Other income (expense), net.

9. Income Taxes

No provision for U.S. federal or state income taxes has been recorded as the Company has incurred net operating losses since inception. Significant components of the Company's net deferred income tax assets as of December 31, 2014 and 2013 consist of the following:

(in thousands)	DECEMBER 31,	
	2014	2013
Net deferred tax assets:		
Tax loss carry-forwards	\$42,863	\$35,428
Other assets	4,882	1,742
Research and development credits	631	556
Other liabilities	(693)	(782)
Valuation allowance	(47,683)	(36,944)
Total net deferred income taxes	\$—	\$—

A reconciliation of the statutory tax rates and the effective tax rates for the years ended December 31, 2014, 2013 and 2012 is as follows:

	YEAR ENDED DECEMBER 31,		
	2014	2013	2012
U.S. federal tax rate	35.00	% 35.00	% 35.00
State tax rate	6.13	% 5.36	% 5.17
Other	(0.04))% (0.03))% (0.02)
Valuation allowance	(41.09))% (40.33))% (40.15)
Effective tax rate	—	% —	% —

Realization of the future tax benefits is dependent on the Company's ability to generate sufficient taxable income within the carry-forward period. Due to the Company's history of operating losses, the deferred tax assets arising from the aforementioned future tax benefits are currently not likely to be realized and, accordingly, are offset by a full valuation allowance. The income tax provision varies from the expected provision determined by applying the federal statutory income tax rate to income (loss). The reasons for the difference in the expected provision, as determined by applying the federal statutory income tax rate to net income (loss) is primarily due to the increase in the deferred income tax valuation allowance of \$10.7 million, \$11.1 million and \$6.1 million for the years ended December 31, 2014, 2013 and 2012, respectively. The net increase in the deferred income tax valuation allowance of \$10.7 million for the year ended December 31, 2014, reflects a reduction of \$2.5 million attributable to the transfer of deferred state tax benefits to an unrelated third party as permitted by the New Jersey Economic Development Authority's sponsored Technology Business Tax Certificate Transfer Program.

As of December 31, 2014, the Company has federal and state net operating loss carry-forwards of approximately \$133.6 million and \$136.6 million, respectively, and federal research and development tax credit carry-forwards of \$0.6 million available to offset federal and state income tax, which expire from 2024 through 2032. Included in the net operating loss carry-forwards are approximately \$8.3 million of net operating loss carry-forwards related to exercises of Non-qualified Stock Options, the tax benefit from which, if realized, will be credited to additional paid-in capital.

Utilization of the net operating loss carry-forwards and credits may be subject to a substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code of 1986, as amended, or the IRC, and similar state provisions. The effect of an ownership change could be an imposition of an annual limitation on the use of net operating loss carry-forwards attributable to periods before the change.

F-16

Table of Contents

10. Convertible Preferred Stock

On October 30, 2013, immediately prior to the closing of the Company's IPO, all outstanding shares of convertible preferred stock automatically were converted into an aggregate 12,120,531 shares of common stock. As of December 31, 2014 and December 31, 2013, the Company does not have any convertible preferred stock authorized, issued or outstanding.

On February 23, 2011, the Company received \$23.8 million in proceeds from the issuance of its Series B Convertible Preferred Stock, net of \$1.2 million of transaction costs.

In connection with the issuance of the 2012 Notes, the Company's certificate of incorporation was amended on December 7, 2012, to authorize the issuance of 16,534,582 shares of preferred stock, of which 400,000 were designated as Series A-1 Preferred Stock; 2,002,006 were designated as Series A-2 Preferred Stock; 4,495,895 were designated as Series A-3 Preferred Stock; 1,136,681 were designated as Series A-4 Preferred Stock; and 8,500,000 were designated as Series B Preferred Stock.

Carrying Value

The convertible preferred stock was originally recorded at the net proceeds received by the Company at issuance. The difference between the net proceeds and the total redemption price was being accreted on a straight-line basis over the period from issuance until the earliest redemption date and was accreted to the convertible preferred stock capital account through the completion of the IPO. Accretion amounted to \$210,000, and \$258,000 for the years ended December 31, 2013 and 2012, respectively.

The Series A-4 Convertible Preferred Stock issued in connection with the conversion of the 2010 Notes on February 23, 2011 was recorded at fair value. The difference between stated and fair value of \$1.3 million was being accreted on a straight-line basis of the period from February 23, 2011 until the earliest redemption date and was accreted to the Series A-4 Convertible Preferred Stock capital account through the completion of the IPO. Accretion amounted to \$240,000 and \$292,000 for the years ended December 31, 2013 and 2012, respectively. The Company determined that the straight-line method approximated the effective interest method.

11. Stockholders' Equity

On October 30, 2013, the Company completed its IPO and issued 7,728,000 shares of its common stock at an IPO price of \$10.00 per share, including 1,008,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares to cover over-allotments. The shares began trading on the NASDAQ Global Market on October 25, 2013. The Company received net proceeds from the IPO of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million. In connection with the IPO, the following events occurred:

• On October 24, 2013, 297,366 warrants to purchase convertible preferred stock were net exercised and were subsequently automatically converted into 297,366 shares of common stock on October 30, 2013 (Note 12);

• On October 30, 2013, 186,248 warrants to purchase convertible preferred stock were net exercised and were subsequently automatically converted into 186,248 shares of common stock on October 30, 2013 (Note 12);

• On October 30, 2013, the outstanding shares of convertible preferred stock were automatically converted into an aggregate 12,120,531 shares of common stock (Note 10);

• On October 30, 2013, 717,801 warrants to purchase convertible preferred stock were automatically converted into 717,801 warrants to purchase common stock, at which time the liabilities were re-measured and reclassified to equity (Note 12);

• On October 30, 2013, the principal and interest outstanding under the Company's 2012 Notes were converted into 1,860,363 shares of common stock at a conversion price equal to the IPO price of \$10.00 per share (Note 8);

• On October 30, 2013, the 2013 Omnibus Incentive Plan became effective under which 3,229,068 equity awards for common stock of the Company may be distributed (Note 13); and

- On October 30, 2013, the 2013 Employee Stock Purchase Plan became effective under which a maximum of 645,814 shares of common stock of the Company may be issued (Note 16).

F-17

Table of Contents

Additionally, on October 30, 2013, the Company's certificate of incorporation was amended to increase the number of authorized shares of common stock to 150,000,000 with a par value of \$0.001 per share and decrease the number of authorized preferred stock to 15,000,000 with a par value of \$0.001 per share.

On November 3, 2014, the Company filed a shelf registration statement on Form S-3, which was declared effective by the SEC on November 10, 2014. The shelf registration statement permits: (i) the offering, issuance and sale by the Company of up to a maximum aggregate offering price of \$150.0 million of the Company's common stock; (ii) sales of common stock by certain selling stockholders; and (iii) the offering, issuance and sale by the Company of up to a maximum aggregate offering price of \$50.0 million of the Company's common stock that may be issued and sold under an "at-the-market" sales agreement with Cantor Fitzgerald & Co. The common stock that may be offered, issued and sold by the Company under the "at-the-market" sales agreement is included in the \$150.0 million of common stock that may be offered, issued and sold by the Company under the shelf registration statement. As of December 31, 2014, the Company has not sold any securities registered pursuant to the shelf registration statement.

Holders of common stock are entitled to dividends when and if declared by the Company's Board of Directors subject to prior rights of the holders of any preferred stock. The holder of each share of common stock is entitled to one vote.

12. Stock Purchase Warrants

During 2006, in connection with a long-term debt agreement, the Company granted warrants to purchase 2,006 shares of Series A-2 Convertible Preferred Stock. The term of the warrants extends ten years from the grant date and the warrants are exercisable at any time during that ten-year period.

In connection with the convertible notes issued in 2012, 2010 and 2009, the Company sold warrants to purchase 1,275,680 shares of Series B, Series A-4 or Series A-3 Convertible Preferred Stock as of December 31, 2013. The term of the warrants issued in 2010 and 2009 extends ten years from the grant date. The term of the warrants issued in 2012 extends seven years from the grant date. All warrants were issued with certain provisions that protect holders from a decline in stock price.

On October 24, 2013, warrants to purchase 297,366 shares of Series B and Series A-3 Convertible Preferred Stock were net exercised and were subsequently automatically converted into 297,366 shares of common stock on October 30, 2013. On October 30, 2013, warrants to purchase 186,248 shares of Series B, Series A-4 and Series A-3 Convertible Preferred Stock were net exercised and were subsequently automatically converted into 186,248 shares of common stock on October 30, 2013.

On October 30, 2013, upon closing of the IPO, the remaining 717,801 outstanding warrants to purchase convertible preferred stock were automatically converted into 717,801 warrants to purchase common stock and all price protection provisions associated with the warrants were removed, at which time the liabilities were revalued and reclassified to equity.

As of December 31, 2014, the following warrants were outstanding:

NUMBER OF UNDERLYING SHARES	EXERCISE PRICE PER SHARE	WARRANT EXPIRATION DATE	TYPE OF EQUITY SECURITY
2,006	\$5.00	March 2016	Common Stock
75,000	\$5.00	February 2019	Common Stock
75,000	\$5.00	November 2019	Common Stock
157,500	\$5.00	August 2020	Common Stock
408,295	\$0.05	December 2019	Common Stock

The warrants outstanding at December 31, 2014 are all currently exercisable with weighted-average remaining lives of 4.99 years.

Prior to the IPO, the Company recognized all of its outstanding warrants as liabilities on its balance sheet as they were subject to price protection provisions. The liability was revalued at each reporting period and changes in the fair value of the warrant liability were included as a component of Other income (expense), net. The initial recognition and

subsequent changes in fair value of the warrant liability had no effect on the Company's cash flows.

F-18

Table of Contents

The Company estimated the fair value of the warrants using the Black-Scholes option-pricing model utilizing the fair value of underlying preferred and common stock. Black-Scholes has inherent limitations for use in the case of a warrant with a price protection provision, since the model is designed to be used when the inputs to the model are static throughout the life of a security. Management concluded, under the Company's facts and circumstances, that the estimated fair values of the warrants using the Black-Scholes option-pricing model approximates, in all material respects, the values determined using a Monte Carlo valuation model. The estimates in the Black-Scholes option-pricing model and the Monte Carlo valuation model are based, in part, on assumptions, including but not limited to stock price volatility, the expected life of the warrants, the risk free rate and the fair value of the equity stock underlying the warrants.

Key assumptions utilized in the fair value calculation as of October 30, 2013, the IPO closing date, and December 31, 2012 appear in the table below.

	OCTOBER 30, 2013	DECEMBER 31, 2012		
Expected term (years)	5.32-6.84	6.13-7.66		
Volatility	65.00	60.00	%	%
Risk-free interest rate	1.51%-2.00%	0.98%-1.31%		
Dividend yield	—	—	%	%

13. Stock-based Compensation

Stock-based compensation expense for options granted, restricted stock, and stock purchase rights are reflected in the statement of operations as follows:

(in thousands)	YEAR ENDED DECEMBER 31,		
	2014	2013	2012
Research and development	\$1,339	\$222	\$89
General and administrative	7,839	2,636	341
Total	\$9,178	\$2,858	\$430

Stock Option Plan

The Company maintains two equity compensation plans, the 2005 Aerie Pharmaceutical Stock Plan (the "2005 Plan") and the 2013 Omnibus Incentive Plan (the "2013 Equity Plan"). The 2005 Plan and the 2013 Plan are referred to collectively as the "Plans." On July 13, 2005, the Company's Board of Directors adopted and approved the 2005 Plan, which, as amended in 2008, 2009, 2011 and 2013, provides for the granting of up to 3,586,277 stock-based awards to employees, directors and consultants of the Company. The Board of Directors determines price, term and vesting conditions of all stock-based awards at their grant date. Absent a public market price for the Company's common stock prior to completion of the IPO, the Board of Directors determined the estimated fair market value for the underlying common stock based on their appraisal of the Company's fair value. Stock-based awards vest over variable periods, generally ranging from one to five years, and expire not more than ten years after the date of grant. On October 30, 2013, the effective date of the 2013 Equity Plan, the 2005 Plan was frozen and no additional awards will be made under the 2005 Plan. Any shares remaining available for future grant under the 2005 Plan were allocated to the 2013 Equity Plan. The 2013 Equity Plan provides for the granting of up to 3,229,068 equity awards for common stock of the Company. As of December 31, 2014, 1,263,100 equity awards have been granted under the 2013 Equity Plan.

Table of Contents

The following table summarizes the stock option activity under the Plans:

	NUMBER OF SHARES	WEIGHTED AVERAGE EXERCISE PRICE
Options outstanding at December 31, 2011	1,152,194	\$ 0.31
Granted	575,200	1.45
Exercised	(12,907) 0.39
Cancelled	(160,287) 0.23
Options outstanding at December 31, 2012	1,554,200	\$ 1.39
Granted	2,009,551	3.11
Exercised	(3,195) 0.37
Cancelled	(370,896) 1.38
Options outstanding at December 31, 2013	3,189,660	\$ 2.16
Granted	1,263,100	20.38
Exercised	(592,044) 0.42
Cancelled	(34,257) 14.32
Options outstanding at December 31, 2014	3,826,459	\$ 8.39
Options exercisable at December 31, 2014	1,329,347	\$ 2.18

The aggregate intrinsic value of options exercised for the years ended December 31, 2014, 2013 and 2012 was \$10.5 million, \$9,000 and \$10,000, respectively. Intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for which the options were exercised.

The following table provides additional information about the Company's stock options that are outstanding and exercisable at December 31, 2014:

EXERCISE PRICE	OPTIONS OUTSTANDING	WEIGHTED AVERAGE EXERCISE PRICE	AGGREGATE INTRINSIC VALUE (000's)	WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE (YEARS)	OPTIONS EXERCISABLE	WEIGHTED AVERAGE EXERCISE PRICE	AGGREGATE INTRINSIC VALUE (000's)
\$0.01 - \$3.15	2,591,459			8.0	1,327,012		
15.55 - 17.97	180,500			9.3	—		
19.07 - 22.31	969,700			9.2	2,335		
24.59 - 25.99	84,800			9.7	—		
	3,826,459	\$8.34	\$ 79,792		1,329,347	\$ 2.18	\$ 35,911

As of December 31, 2014, the Company had \$23.1 million of unrecognized compensation expense related to unvested share options granted under the Plans. This cost is expected to be recognized over a weighted average period of 2.6 years as of December 31, 2014. The weighted average remaining contractual life on all outstanding options as of December 31, 2014 was 8.4 years.

The intrinsic value is calculated as the difference between the estimated fair market value and the exercise price per share of the stock options. The estimated fair market value per share of common stock as of December 31, 2014 and

2013 was \$29.19 and \$17.96, respectively.

The estimated fair value of options granted is determined on the date of grant using the Black-Scholes option pricing model. Options granted to non-employees are revalued at each financial reporting period until required service is performed.

F-20

Table of Contents

Key assumptions utilized in the fair value calculation for the underlying common stock as of December 31, 2014 and December 31, 2013 and 2012 appear on the table below.

	YEAR ENDED DECEMBER 31,				
	2014	2013	2012		
Expected term (years)	6.25	6.25	6.25		
Expected stock price volatility ⁽¹⁾	80.44	% 79.20	% 60.00		%
Risk-free interest rate	1.90	% 1.78	% 1.05		%
Dividend yield	—	% —	% —		%

⁽¹⁾ Weighted Average for 2014

The Company utilized the guidance set forth in the SEC Staff Accounting Bulletin 107, Share-Based Payment (“SAB 107”), to determine the expected term of options. The Company utilized the simplified method as prescribed by SAB 107, as it does not have sufficient historical exercise and post vesting termination data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees. The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities similar to the expected time to liquidity.

Volatility is based on historical volatility of several public entities that are similar to the Company as the Company does not have sufficient historical transactions of its own shares on which to base expected volatility. This peer group of companies utilized in 2014 remained consistent with that of 2013. Other than a one-time dividend in October 2012 related to the spin-off of certain non-core intellectual property rights (Note 15), the Company has not historically issued any dividends and does not expect to in the foreseeable future.

Restricted Common Stock

On March 21, 2013, concurrent with the cancellation of 345,000 stock options, the Company issued 371,034 shares of restricted stock to an employee. The vesting of these awards is time-based with terms of two to four years. These restricted stock awards are subject to repurchase, such that the Company has the right, but not the obligation, to repurchase unvested shares upon the employee’s termination. As of December 31, 2014, 103,064 shares of restricted stock awards were unvested and subject to repurchase.

Compensation expense related to these restricted stock awards is based on the market value of the Company’s common stock on the date of grant and is expensed on a straight-line basis (net of estimated forfeitures) over the vesting period.

The weighted average remaining contractual term for restricted stock awards as of December 31, 2014 was 1.4 years.

Compensation expense related to restricted stock awards for the years ended December 31, 2014 and 2013 was \$390,000 and \$346,000, respectively and was included in general and administrative expense. As of December 31, 2014, the Company had \$242,000 of unrecognized compensation expense, related to unvested restricted stock awards. This cost is expected to be recognized over a weighted average period of 1.4 years as of December 31, 2014.

Stock Purchase Rights

On October 30, 2013, the Company’s 2013 Employee Stock Purchase Plan (the “Purchase Plan”) became effective (Note 16). Compensation expense for stock purchase rights under the Purchase Plan is measured and recognized on the date that the Company becomes obligated to issue shares of common stock and is based on the difference between the fair value of the Company’s common stock and the purchase price on such date. Compensation expense related to stock purchase rights for the years ended December 31, 2014 and 2013 was \$63,000 and \$66,000, respectively.

14. Commitments and Contingencies

Lease Commitment Summary

The following table presents future minimum commitments of the Company due under non-cancelable operating leases with original or remaining terms in excess of one year at December 31, 2014.

Table of Contents

Minimum lease payments were as follows at December 31, 2014:

(in thousands)	
2015	\$460
2016	309
2017	332
2018	328
2019	396
2020 and thereafter	267
Total minimum lease payments	\$2,092

Rent expense was \$579,000, \$403,000 and \$333,000 for the years ended December 31, 2014, 2013 and 2012, respectively, and is reflected in general and administrative expenses and research and development expenses as determined by the underlying activities occurring at each of the Company's locations.

Litigation

The Company is not party to any known litigation, is not aware of any unasserted claims and does not have contingency reserves established for any litigation liabilities.

Contract Service Providers

In the course of the Company's normal business operations, it has agreements with contract service providers to assist in the performance of its research and development, clinical research and manufacturing. Substantially all of these contracts are on an as needed basis.

15. Related-Party Transactions

Prior to their conversion into common stock in connection with the IPO (Note 8), the 2012 Notes were due to holders of the Company's convertible preferred stock. Interest expense on those obligations for the years ended December 31, 2013 and 2012 was \$588,000 and \$16,000, respectively.

In October 2012, the Company formed Novaer Holding, Inc. ("Novaer"), a wholly-owned entity, and contributed certain non-core, non-competitive intellectual property, including an exclusive license for Aerie's intellectual property for non-ophthalmic indications, and \$0.1 million in cash for initial funding. The Board of Directors declared a dividend and distributed 100% of Novaer's equity interests to Aerie's stockholders and warrant holders of record as of September 6, 2012. The book value of the non-core intellectual property was \$0. The cash included in the transaction was recorded as a \$0.1 million cash dividend. Following this spin-off, Novaer is an independent company. Aerie does not own an equity interest and has no significant influence by contract or other means. In addition, Aerie does not retain any rights or obligations and has no share in profits or earnings. On September 6, 2013, the Company terminated its agreement to exclusively license to Novaer its intellectual property for non-ophthalmic indications. No consideration, or future obligation thereof, was exchanged in connection with this termination. As of December 31, 2014, the Company owns all of the worldwide rights to its current product candidates for all indications, both ophthalmic and non-ophthalmic.

For the years ended December 31, 2014, 2013 and 2012, the respective amounts of approximately \$333,000, \$190,000 and \$34,000 were paid to board members, some of whom served as consultants of the Company through consulting agreements containing arm's-length terms typical of agreements entered into with unaffiliated third parties.

Table of Contents

16. Benefit Plan

401 (k) Plan

The Company has adopted a 401(k) deferred compensation plan. Eligible employees meeting the participant criteria may contribute up to the statutory limitation (\$17,500 for each of 2014 and 2013). The Company may contribute a discretionary match if it elects to do so. During the years ended December 31, 2014, 2013 and 2012, the Company contributed \$0 as a matching contribution to the plan.

Employee Stock Purchase Plan

On October 30, 2013, the Company adopted the Purchase Plan under which substantially all employees may purchase the Company's common stock through payroll deductions and lump sum contributions at a price equal to 85% of the lower of the fair market values of the stock as of the beginning or the end of the offering periods. Employees may not purchase more than \$25,000 of stock during any calendar year. As of December 31, 2014, approximately 634,873 shares were reserved for future issuance under the Purchase Plan.

17. Subsequent Events

In January 2015, the Company participated in the New Jersey Economic Development Authority's sponsored Technology Business Tax Certificate Transfer Program to transfer \$3.1 million in state tax benefits to unrelated profitable businesses with operations in the state of New Jersey. The Company received net proceeds of \$2.9 million from the transfer.

In January 2015, the Company entered into a lease agreement (the "Irvine Lease Agreement") under which the Company is leasing approximately 14,500 square feet of office space in Irvine, California. The initial term of the lease expires in January 2021. Total additional rental payments under the Irvine Lease Agreement are approximately \$2.9 million and are expected to be incurred from January 2015 to January 2021.

On February 25, 2015, the Board of Directors authorized grants of common stock options and restricted stock awards to the officers of the Company in the aggregate amount of 363,750 and 60,627, respectively. The options have an exercise price of \$28.03 per share, a term of four years and are subject to vesting conditions over a four-year period. The aggregate fair value of these option grants was \$6.7 million. The restricted stock awards are subject to vesting conditions over a four-year period. The aggregate fair value of these restricted stock awards was \$1.7 million.

18. Selected Quarterly Financial Data (Unaudited)

The following table presents selected unaudited quarterly financial information for the years ended December 31, 2014 and 2013. The results for any quarter are not necessarily indicative of future quarterly results and, accordingly, period to period comparisons should not be relied upon as an indication of future performance.

(in thousands, except share and per share amounts)	FOR THE QUARTER ENDED			
	DECEMBER 31,	SEPTEMBER 30,	JUNE 30,	MARCH 31,
Year Ended December 31, 2014				
Operating expenses	\$(15,973)) \$(13,174)) \$(11,843)) \$(8,982)
Net loss attributable to common stockholders	\$(16,501)) \$(13,147)) \$(11,814)) \$(6,671)
Net loss per share attributable to common stockholders—basic and diluted	\$(0.69)) \$(0.54)) \$(0.49)) \$(0.28)
Year Ended December 31, 2013				
Operating expenses	\$(6,750)) \$(5,686)) \$(5,113)) \$(4,621)
Net loss attributable to common stockholders	\$(10,319)) \$(10,887)) \$(6,465)) \$(3,927)
Net loss per share attributable to common stockholders—basic and diluted	\$(0.62)) \$(10.81)) \$(6.59)) \$(4.07)

Table of Contents

EXHIBIT INDEX

EXHIBIT
NO.

EXHIBIT DESCRIPTION

1.1	Sales Agreement, dated November 3, 2014, by and between Aerie Pharmaceuticals, Inc. and Cantor Fitzgerald & Co. (incorporated by reference to Exhibit 1.1 to the Registrant's Form S-3 Registration Statement (File No. 333-199821) filed on November 3, 2014).
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on October 31, 2013).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on October 31, 2013).
4.1†	Note Purchase Agreement between Aerie Pharmaceuticals, Inc. and Deerfield Partners, L.P., Deerfield International Master Fund, L.P., Deerfield Private Design Fund III, L.P., Deerfield Special Situations Fund, L.P. and Deerfield Special Situations International Master Fund, L.P., dated as of September 8, 2014 (incorporated by reference to Exhibit 4.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-36152) filed on November 12, 2014).
4.2	Form of Note (included in Exhibit 4.1) (incorporated by reference to Exhibit 4.2 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-36152) filed on November 12, 2014).
4.3	Security Agreement among Aerie Pharmaceuticals, Inc. and Deerfield Partners, L.P., Deerfield International Master Fund, L.P., Deerfield Private Design Fund III, L.P., Deerfield Special Situations Fund, L.P. and Deerfield Special Situations International Master Fund, L.P., as Purchasers, and Deerfield Management Company, L.P., as Agent for the Purchasers, dated September 8, 2014 (incorporated by reference to Exhibit 4.3 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-36152) filed on November 12, 2014).
10.1	Amended and Restated Investors' Rights Agreement by and among Aerie Pharmaceuticals, Inc. and TPG Biotechnology Partners, L.P., ACP IV, L.P., Sofinnova Venture Partners VII, L.P., Clarus Lifesciences II, L.P., Osage University Partners I, L.P., Thomas J. van Haarlem, M.D. and Casey Kopczynski, M.D., dated February 23, 2011 (incorporated by reference to Exhibit 10.1 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.2	First Amendment to Amended and Restated Investors' Rights Agreement by and among Aerie Pharmaceuticals, Inc. and TPG Biotechnology Partners, L.P., ACP IV, L.P., Sofinnova Venture Partners VII, L.P., Clarus Lifesciences II, L.P. and Osage University Partners I, L.P., dated December 7, 2012 (incorporated by reference to Exhibit 10.2 to the

Edgar Filing: AERIE PHARMACEUTICALS INC - Form 10-K

Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

10.3 Form of Aerie Pharmaceuticals, Inc. Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.3 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

10.4 Form of Aerie Pharmaceuticals, Inc. Omnibus Incentive Plan (incorporated by reference to Exhibit 10.4 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

10.5 Form of Aerie Pharmaceuticals, Inc. Incentive Stock Option Agreement (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on March 19, 2014).

10.6 Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of July 13, 2005 (incorporated by reference to Exhibit 10.5 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

10.7 First Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of February 19, 2008 (incorporated by reference to Exhibit 10.6 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

10.8 Second Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of December 3, 2009 (incorporated by reference to Exhibit 10.7 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).

Table of Contents

10.9	Third Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of February 23, 2011 (incorporated by reference to Exhibit 10.8 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.10	Fourth Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of August 9, 2013 (incorporated by reference to Exhibit 10.9 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.11	Fifth Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan, dated as of September 16, 2013 (incorporated by reference to Exhibit 10.10 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.12	Separation Agreement and General Release, dated as of July 25, 2013, by and between Aerie Pharmaceuticals, Inc. and Thomas J. van Haarlem (incorporated by reference to Exhibit 10.11 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.13	Consulting Agreement, effective as of July 25, 2013, by and between Aerie Pharmaceuticals, Inc. and Thomas J. van Haarlem (incorporated by reference to Exhibit 10.12 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.14	Form of Indemnification Agreement for officers and directors (incorporated by reference to Exhibit 10.19 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 21, 2013).
10.15	Employment Agreement, dated as of September 20, 2013, by and between Aerie Pharmaceuticals, Inc. and Vicente Anido, Jr., Ph.D. (incorporated by reference to Exhibit 10.18 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.16	Employment Agreement, dated as of July 31, 2013, by and between Aerie Pharmaceuticals, Inc. and Thomas Mitro (incorporated by reference to Exhibit 10.17 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.17	Amended and Restated Employment Agreement, dated as of December 18, 2013, between Aerie Pharmaceuticals, Inc. and Thomas Mitro (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on December 20, 2013).
10.18	Letter Agreement, dated as of September 24, 2012, by and between Aerie Pharmaceuticals, Inc. and Richard Rubino (incorporated by reference to Exhibit 10.13 to the Registrant's Form S-1 Registration Statement (File No. 333-191219) filed on October 3, 2013).
10.19	Amended and Restated Employment Agreement, dated as of December 18, 2013, between Aerie Pharmaceuticals, Inc. and Richard Rubino (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on December 20, 2013).

Edgar Filing: AERIE PHARMACEUTICALS INC - Form 10-K

- 10.20 Amended and Restated Employment Agreement, dated as of December 18, 2013, between Aerie Pharmaceuticals, Inc. and Brian Levy (incorporated by reference to Exhibit 10.3 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on December 20, 2013).
- 10.21 Employment Agreement, dated as of December 18, 2013, between Aerie Pharmaceuticals, Inc. and Casey Kopczynski (incorporated by reference to Exhibit 10.4 to the Registrant's Current Report on Form 8-K (File No. 001-36152) filed on December 20, 2013).
- 23.1* Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm.
- 31.1* Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended.
- 31.2* Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended.
- 32.1* Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
- 32.2* Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
- 101.INS** XBRL Instance Document.
- 101.SCH** XBRL Taxonomy Extension Schema Document.
-

Table of Contents

101.CAL** XBRL Taxonomy Extension Calculation Linkbase Document.

101.LAB** XBRL Taxonomy Extension Label Linkbase Database.

101.PRE** XBRL Taxonomy Extension Presentation Linkbase Document.

101.DEF** XBRL Taxonomy Extension Definition Linkbase Document.

† Certain portions of this exhibit have been omitted and separately filed with the SEC pursuant to a request for confidential treatment which has been granted by the SEC.

* Filed herewith

** Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at December 31, 2014 and 2013, (ii) Statements of Operations and Comprehensive Loss for the years ended December 31, 2014, 2013 and 2012 (iii) Statements of Cash Flows for the years ended December 31, 2014, 2013 and 2012 and (iv) Notes to Financial Statements.