

AERIE PHARMACEUTICALS INC

Form 10-Q

December 05, 2013

[Table of Contents](#)

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2013

or

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

For the transition period from _____ to _____

Commission File Number 001-36152

Aerie Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	20-3109565 (I.R.S. Employer Identification Number)
135 US Highway 206, Suite 15 Bedminster, New Jersey 07921 (908) 470-4320	

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

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

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

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

Large accelerated filer <input type="checkbox"/>	Accelerated filer <input type="checkbox"/>
Non-accelerated filer <input checked="" type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company <input type="checkbox"/>

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

As of November 29, 2013, there were 23,273,626 shares of the registrant's common stock, par value \$0.001, outstanding.

Table of Contents

TABLE OF CONTENTS

	Page
<u>SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS</u>	
<u>PART I. FINANCIAL INFORMATION</u>	2
Item 1. <u>Financial Statements (Unaudited)</u>	
<u>Balance Sheets</u>	2
<u>Statements of Operations and Comprehensive Loss</u>	3
<u>Statements of Cash Flows</u>	4
<u>Notes to the Financial Statements</u>	5
Item 2. <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	12
Item 3. <u>Quantitative and Qualitative Disclosures About Market Risk</u>	21
Item 4. <u>Controls and Procedures</u>	21
<u>PART II. OTHER INFORMATION</u>	22
Item 1. <u>Legal Proceedings</u>	22
Item 1A. <u>Risk Factors</u>	22
Item 2. <u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	54
Item 3. <u>Defaults Upon Senior Securities</u>	54
Item 4. <u>Mine Safety Disclosures</u>	54
Item 5. <u>Other Information</u>	54
Item 6. <u>Exhibits</u>	54

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, continue, estimates, anticipates, plans, intends, may, could, might, will, should or other words that convey uncertainty of future events or circumstances. We 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 clinical trials and anticipated Phase 3 and Phase 2b clinical trials for our current product candidates, including statements regarding the timing of initiation and completion of the 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;

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;

our estimates regarding anticipated capital requirements and our needs for additional financing;

our expectations regarding the clinical effectiveness of our product candidates and results of our clinical trials;

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 ability to protect our proprietary technology and enforce our intellectual property rights;

our expectations related to the use of proceeds from our initial public offering; and

our expectations regarding licensing, acquisitions and strategic operations.

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 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 II, 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. FINANCIAL INFORMATION****Item 1. Financial Statements****AERIE PHARMACEUTICALS, INC.****(A Development Stage Company)****Balance Sheets****(Unaudited)**

(in thousands, except share and per share data)

	September 30, 2013	December 31, 2012
Assets		
Current assets		
Cash and cash equivalents	\$ 4,619	\$ 2,925
Prepaid expenses and other current assets	117	113
Deferred offering costs	2,330	
Total current assets	7,066	3,038
Furniture, fixtures and equipment, net	114	133
Other assets, net	62	48
Total assets	\$ 7,242	\$ 3,219
Liabilities, Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities		
Accounts payable and other current liabilities	\$ 3,175	\$ 1,437
Notes payable, net of discount related parties	14,433	2,331
Interest payable related parties	504	16
Total current liabilities	18,112	3,784
Warrants liability related parties	11,485	2,456
Total liabilities	29,597	6,240

Commitments and contingencies (Note 9)

Convertible preferred stock, \$0.001 par value, 87,872,909 shares authorized as of September 30, 2013 and 82,672,909 shares authorized as of December 31, 2012

Series A-1 2,000,000 shares authorized as of September 30, 2013 and December 31, 2012; 2,000,000 shares issued and outstanding as of	1,000	1,000
--	-------	-------

Edgar Filing: AERIE PHARMACEUTICALS INC - Form 10-Q

September 30, 2013 and December 31, 2012

Series A-2 10,010,029 shares authorized as of September 30, 2013 and December 31, 2012; 10,000,000 shares issued and outstanding as of September 30, 2013 and December 31, 2012	10,000	10,000
Series A-3 22,479,476 shares authorized as of September 30, 2013 and December 31, 2012; 20,979,476 shares issued and outstanding as of September 30, 2013 and December 31, 2012	20,979	20,979
Series A-4 5,683,404 shares authorized as of September 30, 2013 and December 31, 2012; 4,895,904 shares issued and outstanding as of September 30, 2013 and December 31, 2012	4,826	4,606
Series B 47,700,000 shares authorized as of September 30, 2013 and 42,500,000 shares authorized as of December 31, 2012; 22,727,273 shares issued and outstanding as of September 30, 2013 and December 31, 2012	24,506	24,313
Total convertible preferred stock	61,311	60,898
Stockholders' deficit		
Common stock, \$0.001 par value; 22,000,000 shares authorized as of September 30, 2013 and 20,000,000 shares authorized as of December 31, 2012; 1,021,209 and 964,880 shares issued and outstanding at September 30, 2013 (unaudited) and December 31, 2012, respectively;	1	1
Additional paid-in capital	1,123	4
Deficit accumulated during the development stage	(84,790)	(63,924)
Total stockholders' deficit	(83,666)	(63,919)
Total liabilities, convertible preferred stock and stockholders' deficit	\$ 7,242	\$ 3,219

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

Table of Contents**AERIE PHARMACEUTICALS, INC.****(A Development Stage Company)****Statements of Operations and****Comprehensive Loss****(Unaudited)**

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,		Period From Inception (June 22, 2005) to September 30, 2013
	2013	2012	2013	2012	
Operating expenses					
General and administrative	\$ (3,287)	\$ (1,416)	\$ (6,693)	\$ (3,701)	\$ (26,590)
Research and development	(2,399)	(1,373)	(8,727)	(7,305)	(51,876)
Loss from operations	(5,686)	(2,789)	(15,420)	(11,006)	(78,466)
Other income (expense) net	(5,062)	(803)	(5,446)	(427)	(6,188)
Net loss	\$ (10,748)	\$ (3,592)	\$ (20,866)	\$ (11,433)	\$ (84,654)
Comprehensive loss	\$ (10,748)	\$ (3,592)	\$ (20,866)	\$ (11,433)	\$ (84,654)
Net loss attributable to common stockholders basic and diluted	\$ (10,887)	\$ (3,730)	\$ (21,279)	\$ (11,845)	
Net loss per share attributable to common stockholders basic and diluted	\$ (10.81)	\$ (3.87)	\$ (21.61)	\$ (12.38)	
Weighted average number of common shares outstanding basic and diluted	1,006,893	964,880	984,727	957,079	

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

Table of Contents**AERIE PHARMACEUTICALS, INC.****(A Development Stage Company)****Statements of Cash Flows****(Unaudited)**

(in thousands, except share and per share data)

	Period From	
	Inception	
	(June 22, 2005) to	
	September 30,	
	2013	
	2012	
	2013	
Cash flows from operating activities		
Net loss	\$ (20,866)	\$ (11,433)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	47	104
Amortization and accretion costs related to notes payable related parties	2,377	3,774
Gain on conversion of notes payable		(821)
Stock-based compensation	1,531	292
Interest payable related parties	488	1,625
Change in fair value measurements	3,850	441
Changes in operating assets and liabilities		
Prepaid, current and other assets	(18)	39
Accounts payable and other current liabilities	1,025	(1,395)
Net cash used in operating activities	(11,566)	(11,952)
Cash flows from investing activities		
Purchase of furniture, fixtures and equipment	(28)	(51)
Net cash provided by (used in) investing activities	(28)	(51)
Cash flows from financing activities		
Proceeds from sale of preferred stock		45,000
Payments of stock issuance costs		(1,216)
Proceeds from notes payable to related parties	15,000	34,778
Dividends paid		(130)
Payments of debt issuance costs		(115)

Proceeds from sale of common stock			3
Proceeds from exercise of stock options	1	6	16
Payments of long-term debt			(528)
Payments of initial public offering costs	(1,713)		(1,713)
Net cash provided by financing activities	13,288	6	76,095
Net change in cash and cash equivalents	1,694	(11,997)	4,619
Beginning of period	2,925	15,068	
End of period	\$ 4,619	\$ 3,071	\$ 4,619

Supplemental disclosures**Noncash financing activities**

Conversion of long-term debt into preferred stock			17,364
Debt discount attributable to warrants	5,275		7,724
Accretion from conversion of note payable to related parties	220	219	755
Accretion of stock issuance costs	193	193	722
Deferred offering costs	617		617

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

Table of Contents

AERIE PHARMACEUTICALS, INC.

(A Development Stage Company)

Notes to the Financial Statements

(Unaudited)

1. The Company

Aerie Pharmaceuticals, Inc. (the Company) is a development 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 corporate headquarters in Bedminster, New Jersey, conducts research in Research Triangle Park, North Carolina, and has an office in Newport Beach, California. All technology of the Company is based on own use research and development.

To date, the Company is in the development stage since it has not yet commenced primary operations or generated significant revenue. The Company's activities since inception primarily consisted of developing product and technology rights, 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 accompanying financial statements have been prepared on a basis that assumes the Company will continue as a going concern, which contemplates the realization of assets and the satisfaction of liabilities and commitments in the normal course of business. The Company has funded its operations as of September 30, 2013 primarily through the sale of convertible preferred stock and issuance of convertible notes. In October 2013, the Company completed its initial public offering and issued 6,720,000 shares of its common stock at an initial offering price of \$10.00 per share (Note 11). In addition, the Company sold an additional 1,008,000 shares of common stock directly to its underwriters when they exercised their over-allotment option in full at the initial offering price of \$10.00 per share. The Company received net proceeds from the initial public offering of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million. Including the net proceeds from the initial public offering, the Company estimates that it has sufficient funding to sustain operations through approximately mid-2016. Accordingly, the Company will 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 interim financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (U.S. GAAP). In the opinion of management, the Company has made all adjustments, which include normal recurring adjustments necessary for a fair statement of the Company's financial position and results of operations for the interim periods presented. Certain information and disclosures normally

included in the annual financial statements prepared in accordance with U.S. GAAP have been condensed or omitted. These interim financial statements should be read in conjunction with the audited financial statements and accompanying notes for the year ended December 31, 2012 included in the Company's final prospectus dated October 24, 2013 filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission. The results for the three and nine months ended September 30, 2013 are not necessarily indicative of the results to be expected for a full year, any other interim periods or any future year or period.

Additionally, the Company closed the aforementioned initial public offering in October 2013 as further described in Note 11 to the financial statements. As a result, there have been significant changes to the Company's capital structure subsequent to the date of these financial statements.

Table of Contents

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.

Reverse Stock Split

The Company effected a 1-for-5 reverse stock split of its common stock on October 8, 2013. Accordingly, all share and per share amounts related to common stock and options for all periods presented in these financial statements and notes thereto, have been adjusted retroactively to reflect this reverse stock split. The Company's preferred stock was not subject to the reverse stock split.

Equity Issuances in the Quarter Ended September 30, 2013

In the context of its initial public offering, the Company determined that the probability of the conversion of preferred stock into common stock, based on the consent from the holders of the requisite number of preferred shares, was high as of September 30, 2013. As a result, the allocation of the determined equity value assumed conversion of all preferred stock into common stock. For financial reporting purposes, based on recommendations from management and taking into account advice and assistance provided by third-party valuation consultants engaged to assist in such valuations, the Company's board of directors determined that the fair value of its common stock for all equity transactions during the quarter ended September 30, 2013 and all transactions that require fair value measurement as of September 30, 2013 was consistent with the initial public offering price of \$10.00. Accordingly, for the quarter ended September 30, 2013, the Company recognized a stock-based compensation charge of \$0.2 million related to stock options granted on August 26, 2013 and September 12, 2013 and \$0.7 million related to the remeasurement of grants to non-employees. The total unrecognized stock-based compensation expense related to the August and September 2013 option grants was \$13.6 million and is expected to be recognized ratably through 2017, which represents the expected vesting period of the options (Note 8). In addition, the Company measured the stock purchase warrants issued on August 9, 2013 and September 30, 2013 using the initial public offering price as the deemed fair value of its common stock, resulting in an initial measurement of the warrant liability of \$2.0 million and \$1.4 million, respectively (Note 7).

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.

The Company's material financial instruments consist primarily of cash and cash equivalents, other current assets, accounts payable, accrued expenses, long-term debt and stock purchase warrant liabilities. The fair value of cash and cash equivalents, other current assets, accounts payable and accrued expenses approximate their respective carrying values due to the short-term nature of these instruments. The Company has determined its stock purchase warrants liability to be Level 3 fair value measurement (Note 7).

Recent Accounting Pronouncements

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 will be effective for the Company for interim and annual periods beginning after December 15, 2013, with early adoption permitted. The adoption of this standard is not expected to 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 statement 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 as the Company's net income is equal to its comprehensive income.

In June 2011, the FASB issued amended guidance intended to increase the prominence of items reported on other comprehensive income (loss). This amended guidance requires that all non-owner changes in stockholders' equity (deficit) be presented in a single continuous statement of comprehensive income (loss) or in two separate but consecutive statements. The amended guidance became effective for periods beginning after

Table of Contents

December 15, 2011. The Company has applied this guidance beginning with its financial information for the year ended December 31, 2012. This amended guidance affects presentation, but does not have a material effect on the Company's financial statements.

Net Loss per Common Share

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 potential common stock instruments. Net loss attributable to common stockholders is calculated by adjusting the Company's net loss for accretion on convertible preferred stock (Note 6). Diluted net loss per share attributable to common stock (Diluted EPS) gives 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, which include convertible preferred stock, stock options, notes payable to related parties and stock purchase warrants, have been excluded from the computation of diluted net loss per common share attributable to common stockholders as their inclusion would have the effect of reducing the net loss per common share. Therefore, the denominator used to calculate both basic and diluted net loss per common stock is the same in all periods presented. The Company's potential common stock equivalents that have been excluded from the computation of diluted net loss per share attributable to common stockholders for all periods presented because of their antidilutive effect consist of the following:

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2013	2012	2013	2012
Convertible preferred stock	60,602,653	60,602,653	60,602,653	60,602,653
Outstanding stock options	3,189,660	1,209,200	3,189,660	1,209,200
Notes and interest payable to related parties ⁽¹⁾	\$ 18,504,000	\$	\$ 18,504,000	\$
Stock purchase warrants	6,388,431	2,297,529	6,388,431	2,297,529
Unvested restricted common stock awards	317,900		317,900	

- (1) The 2012 Notes and accrued interest thereon are convertible into capital stock at the option of the holders according to the terms of the 2012 Note and Warrant Agreement. See Note 5.

3. Other Income (Expense), Net

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

	THREE MONTHS ENDED		NINE MONTHS ENDED		PERIOD FROM
	SEPTEMBER 30,		SEPTEMBER 30,		INCEPTION
(in thousands)	2013	2012	2013	2012	(JUNE 22, 2005) TO

**SEPTEMBER 30,
2013**

Interest expense	\$ (1,477)	\$ (15)	\$ (2,865)	\$ (15)	\$ (5,399)
Gain on conversion of notes payable to related parties					821
Sale of New Jersey state tax benefit			1,268		1,268
(Expense)/income due to change in fair value measurements ⁽¹⁾	(3,585)	(788)	(3,850)	(441)	(3,851)
Other income, net			1	29	973
	\$ (5,062)	\$ (803)	\$ (5,446)	\$ (427)	\$ (6,188)

- (1) Includes change in fair value of warrant liabilities and change in fair value of a certain conversion feature related to the 2012 Notes that was determined to be an embedded derivative requiring bifurcation and separate accounting. See Note 7 and Note 5, respectively.

Table of Contents**4. Accounts Payable & Other Current Liabilities**

Accounts payable and other current liabilities consist of the following:

(in thousands)	SEPTEMBER 30, 2013	DECEMBER 31, 2012
Accounts payable	\$ 1,126	\$ 174
Employee benefits and compensation related accruals ⁽¹⁾	1,051	400
Accrued expenses and other liabilities	998	863
	\$ 3,175	\$ 1,437

(1) Comprised of accrued bonus, accrued vacation and accrued severance liabilities.

5. Notes Payable

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 accrue interest at a rate of 8% per annum, with principal plus accrued interest thereon due upon maturity at September 30, 2013. The 2012 Notes are convertible into capital stock at the option of the holders upon the closing of an equity financing that raises at least \$15.0 million, a qualified initial public offering, liquidation or any reorganization, consolidation or merger. The Company may, in its discretion, request a subsequent closing when its cash and cash equivalents balance drops below \$1.5 million. 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 convertible notes to related parties, resulting in an aggregate principal amount of \$18.0 million being authorized. Additionally, 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. The Company classified all convertible notes and related accrued interest as current obligations as of September 30, 2013 and December 31, 2012.

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 recorded the embedded derivative liability within accounts payable and other current liabilities and the change in fair value as a component of Other income (expense), net.

As of September 30, 2013 and December 31, 2012, the Company recognized unamortized debt discounts of \$3.6 million and \$669,000, respectively, relating to the detachable warrants issued in conjunction with the 2012 Notes (Note 7). Debt discounts are amortized using the effective interest method through the earlier of the date of maturity or the conversion of the debt. For the three months and nine month ended September 30, 2013, amortization of debt discounts and accrued interest expense amounted to \$1.5 million and \$2.9 million, respectively.

Table of Contents

6. Convertible Preferred Stock

Concurrent with this issuance of its Series B Convertible Preferred Stock in February 2011, the certificate of incorporation was amended to authorize the issuance of 15,734,582 shares of common stock and 63,672,909 shares of convertible preferred stock, of which 2,000,000 are designated as Series A-1 Convertible Preferred Stock; 10,010,029 are designated as Series A-2 Convertible Preferred Stock; 23,266,976 are designated as Series A-3 Convertible Preferred Stock; 4,895,904 are designated as Series A-4 Convertible Preferred Stock; and 23,500,000 are designated as Series B Convertible Preferred Stock.

In connection with the issuance of the 2012 Notes, the Certificate of Incorporation was amended on December 7, 2012, to authorize the issuance of 20,000,000 shares of common stock and 82,672,909 shares of preferred stock, of which 2,000,000 are designated as Series A-1 Preferred Stock; 10,010,029 are designated as Series A-2 Preferred Stock; 22,479,476 are designated as Series A-3 Preferred Stock; 5,683,404 are designated as Series A-4 Preferred Stock; and 42,500,000 are designated as Series B Preferred Stock.

Concurrent with the amendment to the 2012 Notes, the certificate of incorporation was amended to authorize the issuance of 22,000,000 shares of common stock and 90,172,909 shares of preferred stock, of which 2,000,000 are designated as Series A-1 Preferred Stock; 10,010,029 are designated as Series A-2 Preferred Stock; 22,479,476 are designated as Series A-3 Preferred Stock; 5,683,404 are designated as Series A-4 Preferred Stock; and 50,000,000 are designated as Series B Preferred Stock.

On September 16, 2013, the certificate of incorporation was amended to reflect the re-designation of 2,300,000 unissued shares of Series B Convertible Preferred Stock to common 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 is being accreted on a straight-line basis over the period from issuance until the earliest redemption date. Accretion amounted to \$65,000 and \$193,000 for the three months and nine months ended September 30, 2013, respectively, and \$65,000 and \$193,000 for the three months and nine months ended September 30, 2012, respectively, and \$722,000 for the period from inception (June 22, 2005) to September 30, 2013, respectively.

The Series A-4 Convertible Preferred Stock issued in connection with the conversion of the 2010 Notes (Note 5) in February 2011 was recorded at fair value. The difference between stated and fair value of \$1.3 million is being accreted on a straight-line basis of the period from February 23, 2011 until the earliest redemption date. Accretion amounted to \$74,000 and \$220,000 for the three months and nine months ended September 30, 2013, respectively, and \$73,000 and \$219,000 for the three months and nine months ended September 30, 2012, respectively, and \$755,000 for the period from February 23, 2011 to September 30, 2013, respectively. The Company determined that the straight-line method approximates the effective interest method.

7. Stock Purchase Warrants

In connection with the issuance of long-term debt and convertible notes, the Company granted and/or sold warrants to purchase 6,388,431 and 2,979,345 shares of convertible preferred stock as of September 30, 2013 and December 31, 2012, respectively. All warrants become automatically exercisable to common stock upon a qualified initial public offering or the conversion of the related convertible preferred stock (Note 11).

The Company recognizes all of its warrants with in its balance sheet as liabilities. The liability is revalued at each reporting period and changes in the fair value of the warrant liability are included as a component of Other income (expense), net. The initial recognition and subsequent changes in fair value of the warrant liability have no effect on the Company's cash flows.

Table of Contents

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

	SEPTEMBER 30, 2013		DECEMBER 31, 2012	
Expected term (years)	5.39	6.91	6.13	7.66
Volatility	65.00%		60.00%	
Risk-free interest rate	1.51%	2.00%	0.98%	1.31%
Dividend yield	0%		0%	

For the three months and nine months ended September 30, 2013, the Company recorded \$3.5 million and \$3.8 million in Other income (expense), net, respectively, to reflect the change in fair value. For the three months and nine months ended September 30, 2012, the Company recorded \$788,000 and \$441,000 in Other income (expense), net, respectively, to reflect the change in fair value.

8. Stock-based Compensation

On July 13, 2005, the Company's board of directors adopted and approved the 2005 Aerie Pharmaceutical Stock Plan (the Plan), which, as amended in 2008, 2009, 2011 and 2013, provides for the granting of up to 3,586,227 stock-based awards to employees, directors and consultants of the Company. 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. The Company granted stock options to employees to purchase 2,009,551 and 230,200 shares of common stock for the nine months ended September 30, 2013 and 2012, respectively.

	NUMBER OF SHARES	WEIGHTED AVERAGE EXERCISE PRICE	AGGREGATE INTRINSIC VALUE (000 \$)
Options outstanding at December 31, 2012	1,554,200	\$ 1.3890	\$ 2,348
Granted	2,009,551	3.1121	
Exercised	(3,195)	0.3722	
Cancelled	(370,896)	1.3844	
Options outstanding at September 30, 2013	3,189,660	\$ 2.1634	\$ 24,996
Options exercisable at September 30, 2013	975,669	\$ 0.5911	\$ 9,180

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 re-measured at each financial reporting period until required service is performed.

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

	Period From Inception (June 22, 2005) to				
	Three Months Ended		Nine Months Ended		
(in thousands)	September 30, 2013	September 30, 2012	September 30, 2013	September 30, 2012	September 30, 2013
Research and development	\$ 62	\$ 22	\$ 105	\$ 66	\$ 284
General and administrative	1,068	86	1,426	226	2,277
Total ⁽¹⁾	\$ 1,130	\$ 108	\$ 1,531	\$ 292	\$ 2,561

- (1) For the three and nine months ended September 30, 2013, stock-based compensation expense includes \$0.9 million of expense related to a stock-based compensation charge on the August and September 2013 option grants based on the assessment of certain assumptions utilized in determining the fair value of common stock in the context of the Company's initial public offering (Note 2).

Table of Contents

As of September 30, 2013, the Company had \$14.4 million of unrecognized compensation expense related to options granted under the Plan. This cost is expected to be recognized over a weighted average period of 3.5 years as of September 30, 2013. The weighted average remaining contractual life on all outstanding options as of September 30, 2013 was 6.0 years.

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 September 30, 2013, 317,900 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 September 30, 2013 was 2.3 years. Compensation expense related to restricted stock awards for the three months and nine months ended September 30, 2013 was \$105,000 and \$247,000, respectively and was included in general and administrative expense.

9. Commitments and Contingencies

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.

10. Related-Party Transactions

The notes issued in 2012 are due to holders of the Company's convertible preferred stock. Interest expense on those obligations for the three months and nine months ended September 30, 2013 was \$266,000 and \$488,000, respectively, and is classified as a current obligation on the Company's balance sheets (Note 5).

On September 6, 2013, the Company terminated its agreement to exclusively license to Novaer the Company's intellectual property for non-ophthalmic indications. As of September 6, 2013, the Company owns all of the worldwide rights to the Company's current product candidates for all indications, both ophthalmic and non-ophthalmic.

11. Subsequent Events

On October 8, 2013, the Company effected a 1-for-5 reverse split of its common stock. All share and per share amounts related to common stock and options included in these financial statements and notes to financial statements have been restated to reflect the reverse stock split of the Company's common stock. The Company's preferred stock was not subject to the reverse stock split and, accordingly, the conversion ratios of the Company's preferred stock have been adjusted to reflect the reverse split.

As of October 11, 2013, the Company had obtained a written consent from the holders of its outstanding convertible notes in which the holders agreed to convert all principal and interest accrued thereon to common stock upon the completion of the Company's initial

Table of Contents

public offering at a conversion price equal to the per share offering price. In addition, the Company had obtained the consent from the holders of the requisite number of preferred shares to convert to shares of the Company's common stock upon the completion of the Company's initial public offering.

On October 30, 2013, the Company completed its initial public offering and issued 6,720,000 shares of its common stock at an initial offering price of \$10.00 per share. In addition, the Company sold an additional 1,008,000 shares of common stock directly to its underwriters when they exercised their over-allotment option in full at the initial offering price of \$10.00 per share. The Company received net proceeds from the initial public offering 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 initial public offering, the following events occurred subsequent to September 30, 2013:

On October 24, 2013, 1,486,830 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;

On October 30, 2013, 931,240 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;

On October 30, 2013, the outstanding shares of convertible preferred stock automatically converted into an aggregate 12,120,531 shares of common stock;

On October 30, 2013, the principal and interest outstanding under our \$18.0 million in aggregate principal amount of our 8% convertible notes due December 31, 2013 converted into 1,860,363 shares of common stock at a conversion price equal to the initial public offering price of \$10.00 per share;

On October 30, 2013, 3,589,005 warrants to purchase convertible preferred stock were converted into 717,801 warrants to purchase common stock, at which time the liabilities were re-measured and reclassified to equity.

On October 30, 2013, the 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 October 30, 2013, the Company's By-Laws were amended and restated in their entirety.

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.

On October 30, 2013, the 2013 Employee Stock Purchase Plan became effective under which a maximum of 645,814 shares may be issued.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis should be read in conjunction with our unaudited financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and related notes for the year ended December 31, 2012, included in our prospectus dated October 24, 2013 (the "IPO Prospectus"), filed with the U.S. Securities and Exchange Commission pursuant to Rule 424(b) under the Securities Act.

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, dual-action AR-13324, recently completed a Phase 2b clinical trial in patients with open-angle glaucoma and ocular hypertension. We are also developing a second product candidate, once-daily, triple-action PG324, which is a fixed-dose combination of AR-13324 and latanoprost, the most commonly prescribed drug for the treatment of patients with glaucoma. We are focused on glaucoma because we believe our product candidates provide important new opportunities to improve the treatment of the disease.

We are developing AR-13324 as the first of a new class of compounds that is designed to lower intraocular pressure, or IOP, in patients through a novel dual mechanism of action, or MOA. PG324 is designed to lower IOP through all three MOAs: increasing fluid outflow through the trabecular meshwork, or the TM, the eye's primary drain, increasing fluid outflow through the uveoscleral pathway, the eye's secondary drain, and reducing fluid production in the eye.

Table of Contents

We are a development stage company and have incurred net losses since our inception in June 2005. Our operations to date have been limited to research and development and raising capital. Through September 30, 2013, we have raised net cash proceeds of \$78.6 million from the sale of \$43.8 million of convertible preferred stock and \$34.8 million of convertible notes. Subsequent to their issuance, \$16.2 million of convertible notes converted into shares of convertible preferred stock and \$0.5 million in cash payments were made. To date, we have not generated any revenue. As of September 30, 2013, we have primarily financed our operations through the private placement of our equity securities and issuance of convertible promissory notes. As of September 30, 2013, we had a deficit accumulated during the development stage of \$84.8 million. We recorded net losses of \$10.7 million and \$20.9 million during the three months and nine months ended September 30, 2013, respectively, and net losses of \$3.6 million and \$11.4 million during the three months and nine months ended September 30, 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.

We expect our research and development expenses to increase if and when we initiate Phase 3 and Phase 2b clinical trials for our AR-13324 and PG324 product candidates, respectively, and pursue regulatory approval. As we prepare for commercialization, we will likely incur significant commercial, sales, marketing and outsourced manufacturing expenses. We also expect to incur additional expenses associated with operating as a public company, as a consequence of our initial public offering and listing of our common stock on the NASDAQ Global Market, completed in October 2013. As a result, we expect to continue to incur significant and increasing operating losses at least for the next several years. 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.

On October 30, 2013, we completed our initial public offering and issued 7,728,000 shares of our common stock at an initial offering 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 initial public offering of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million.

We anticipate that we will use approximately \$37.4 million of the net proceeds from the offering for direct clinical and non-clinical costs associated with the completion of Phase 3 registration trials and filing of a New Drug Application with the Food and Drug Administration for our AR-13324 product candidate and approximately \$9.8 million for direct clinical and non-clinical costs associated with the completion of the Phase 2b clinical trial and Phase 3 enabling activities for our PG324 product candidate. We intend to use the remainder of the proceeds of the offering for working capital and general corporate purposes. We expect that these funds will not be sufficient to enable us to complete all necessary development or commercially launch these product candidates. Accordingly, we will 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 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.

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 and legal services. We expect that our general and administrative expenses will increase with the continued

Table of Contents

advancement of our product candidates and with the increased management, legal, compliance, accounting and investor relations expenses we will have as we continue to operate as a public company. We expect these increases will likely include increased expenses for insurance, expenses related to the hiring of additional personnel and payments to outside consultants, 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 CROs, contract manufacturing organizations and consultants 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 salaries, stock-based compensation and depreciation are not allocated to direct clinical or non-clinical expenses and are separately classified as unallocated.

The following table shows our research and development expenses by type of activity for the three months and nine months ended September 30, 2013 and 2012:

THREE MONTHS		NINE MONTHS	
ENDED SEPTEMBER 30,		ENDED SEPTEMBER 30,	
2013	2012	2013	2012
(in thousands)			

Edgar Filing: AERIE PHARMACEUTICALS INC - Form 10-Q

AR-13324:				
Direct non-clinical	\$ 1,139	\$ 474	\$ 2,040	\$ 2,037
Direct clinical	32	170	1,333	700
Total	\$ 1,171	\$ 644	\$ 3,373	\$ 2,737
PG324:				
Direct non-clinical	\$ 209	\$	\$ 209	\$
Direct clinical				
Total	\$ 209	\$	\$ 209	\$
Discontinued product candidates:				
Direct non-clinical	\$ 72	\$ 210	\$ 537	\$ 1,629
Direct clinical	395	84	2,969	1,179
Total	\$ 467	\$ 294	\$ 3,506	\$ 2,808
Unallocated	552	435	1,639	1,760
Total research and development expense	\$ 2,399	\$ 1,373	\$ 8,727	\$ 7,305

Table of Contents

From inception through September 30, 2013, we did not incur any direct non-clinical or direct clinical costs for AR-13533. Costs for this product candidate were primarily comprised of internal employee salaries and were included in unallocated costs. Discontinued product candidates relate to previously developed AR-12286 and related compounds, as they 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 initiate Phase 3 and Phase 2b 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.

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 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.

Table of Contents

Other expense consists of interest accrued under existing convertible notes, amortization of debt discounts and non-cash expense related to changes in the fair value of our warrants liability arising from the stock purchase warrants described in Note 3 to our unaudited financial statements appearing elsewhere in this report.

Accretion of Convertible Preferred Stock

Shares of our convertible preferred stock were initially recorded on our balance sheet at their cost, less associated issuance costs. Series A-1, A-2 and A-3 of our convertible preferred stock are fully accreted as of December 31, 2012.

Our Series A-4 Convertible Preferred Stock issued on February 23, 2011, resulting from the conversion of the notes issued in 2010, was recorded at fair value. The difference between redemption and initial carrying value of \$1.3 million is being ratably accreted over the period from February 23, 2011 until the earliest redemption date, which is August 17, 2015.

Our Series B Convertible Preferred Stock issued on February 23, 2011 was recorded at fair value net of \$1.2 million of issuance costs, which is being ratably accreted over the period from February 23, 2011 until the earliest redemption date, which is August 17, 2015.

The composition of our convertible preferred stock is further described in Note 6 to our unaudited financial statements appearing elsewhere in this report.

Debt Discounts

Our notes payable were issued with warrant coverage. We recorded notes payable on our balance sheet net of a discount equal to the estimated fair value of the associated warrant instrument. The discount is amortized ratably through interest expense over the term of the associated notes.

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.

Equity Issuances in the Quarter Ended September 30, 2013

In the context of our initial public offering, we determined that the probability of the conversion of preferred stock into common stock, based on the consent from the holders of the requisite number of preferred shares, was high as of September 30, 2013. As a result, the allocation of the determined equity value assumed conversion of all preferred stock into common stock. For financial reporting purposes, based on recommendations from management and taking into account advice and assistance provided by third-party valuation consultants engaged to assist in such valuations, our board of directors determined that the fair value of our common stock for all equity transactions during the quarter

ended September 30, 2013 and all transactions that require fair value measurement as of September 30, 2013 was consistent with the initial public offering price of \$10.00. Accordingly, for the quarter ended September 30, 2013, we recognized a stock-based compensation charge of \$0.2 million related to stock options granted on August 26, 2013 and September 12, 2013 and \$0.7 million related to the re-measurement of grants to non-employees. The total unrecognized stock-based compensation expense related to the August and September 2013 option grants was \$13.6 million and is expected to be recognized ratably through 2017, which represents the expected vesting period of the options (see Note 8 to our unaudited financial statements appearing elsewhere in this report). In addition, we measured the stock purchase warrants issued on August 9, 2013 and September 30, 2013 using the initial public offering price as the deemed fair value of our common stock, resulting in an initial measurement of the warrant liability of \$2.0 million and \$1.4 million, respectively (see Note 7 to our unaudited financial statements appearing elsewhere in this report).

Table of Contents

Our significant accounting policies are more fully described in Note 2 to our unaudited financial statements appearing elsewhere in this report and Note 2 to our audited financial statements included in the IPO Prospectus.

Results of Operations***Comparison of the three months ended September 30, 2013 and 2012***

The following table summarizes the results of our operations for the three months ended September 30, 2013 and 2012:

	THREE MONTHS ENDED		%	
	SEPTEMBER 30,	SEPTEMBER 30,	INCREASE	INCREASE
	2013	2012	(DECREASE)	(DECREASE)
	(unaudited)			
	(in thousands)			
Expenses				
General and administrative	\$ (3,287)	\$ (1,416)	\$ 1,871	132%
Research and development	(2,399)	(1,373)	1,026	75%
Other financial income (expense), net	(5,062)	(803)	4,259	530%
Net loss	\$ (10,748)	\$ (3,592)		

General and administrative expenses

General and administrative expenses increased by \$1.9 million for the three months ended September 30, 2013 as compared to the three months ended September 30, 2012. This increase was primarily attributable to an increase of \$1.4 million in personnel costs, including new salaried employees and related employee stock-based compensation expense resulting in part from the hiring of a Chief Financial Officer in October 2012 and a President and Chief Operating Officer in August 2013. Accounting, legal and consulting fees increased by \$0.5 million as a result of increased audit fees, intellectual property and patent expenses and other business related activities.

Research and development expenses

Research and development expenses increased by \$1.0 million for the three months ended September 30, 2013 as compared to the three months ended September 30, 2012. This increase was primarily due to higher direct non-clinical costs of \$0.7 million and higher direct clinical costs of \$0.2 million. The remaining difference was due to the change in unallocated expenses including employee salary and related expenses. Direct non-clinical costs for AR-13324 and PG324 increased \$0.7 million and \$0.2 million, respectively. These increases were offset by a decrease of \$0.2 million in direct non-clinical costs related to the timing of manufacturing requirements for product candidates where further advancement for the treatment of glaucoma was discontinued during the second quarter of 2013. These product candidates did not demonstrate the efficacy endpoint required for further advancement. The increase in direct clinical costs was attributable to higher clinical costs of \$0.3 million related to Phase 2b clinical trials for discontinued product candidates, as previously described, offset by a decrease in direct clinical costs for AR-13324 of \$0.1 million.

Other income (expense), net

Other income (expense), net increased by \$4.3 million for the three months ended September 30, 2013 as compared to the three months ended September 30, 2012. The increase was mainly due to a \$1.5 million increase in non-cash interest expense relating to the amortization of debt discounts and accrued interest and \$2.8 million unfavorable non-cash change in the fair value of warrant liabilities and the fair value of a certain conversion feature related to our convertible notes issued under a Note and Warrant Purchase Agreement dated as of December 7, 2012 (the 2012 Notes) that was determined to be an embedded derivative requiring bifurcation and separate accounting.

Table of Contents***Comparison of the nine months ended September 30, 2013 and 2012***

The following table summarizes the results of our operations for the nine months ended September 30, 2013 and 2012:

	NINE MONTHS ENDED		%	
	SEPTEMBER 30,	SEPTEMBER 30,	INCREASE	INCREASE
	2013	2012	(DECREASE)	(DECREASE)
	(unaudited)			
	(in thousands)			
Expenses				
General and administrative	\$ (6,693)	\$ (3,701)	\$ 2,992	81%
Research and development	(8,727)	(7,305)	1,422	19%
Other financial income (expense), net	(5,446)	(427)	5,019	1,175%
Net loss	\$ (20,866)	\$ (11,433)		

General and administrative expenses

General and administrative expenses increased by \$3.0 million for the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012. This increase was primarily attributable to an increase of \$2.0 million in personnel costs, including new salaried employees, employee stock-based compensation expense resulting in part from the hiring of a Chief Financial Officer in October 2012 and a President and Chief Operating Officer in August 2013. Accounting, legal and consulting fees increased by \$1.0 million as a result of increased audit fees, intellectual property and patent expenses and other business related activities.

Research and development expenses

Research and development expenses increased by \$1.4 million for the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012. The net increase was primarily due to higher direct clinical costs of \$2.4 million offset by a decrease in direct non-clinical costs of \$0.9 million. The remaining difference is due to the change in unallocated expenses including employee salary and related expenses. The direct clinical costs for AR-13324 increased by \$0.6 million due to activity related to its Phase 2b clinical trial. The remaining portion of the increase is attributable to the Phase 2b clinical trials for product candidates where further advancement for the treatment of glaucoma was discontinued during the second quarter of 2013. These products did not meet efficacy requirements for further advancement. The decrease in direct non-clinical costs is attributable to a decline in manufacturing and testing for discontinued product candidates, as previously described, offset by an increase in direct non-clinical costs for PG324 of \$0.2 million due to activity related to manufacturing and testing in preparation for its Phase 2b clinical trial. Direct non-clinical costs for AR-13324 remained consistent for the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012 due to the timing of activities related to Phase 2b and Phase 3 enabling testing.

Other income (expense), net

Other income (expense), net increased by \$5.0 million for the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012. The increase was mainly due to a \$2.9 million increase in non-cash

interest expense relating to the amortization of debt discounts and accrued interest and \$3.4 million unfavorable non-cash change in the fair value of warrant liabilities and the fair value of a certain conversion feature related to the 2012 Notes that was determined to be an embedded derivative requiring bifurcation and separate accounting. 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.

Table of Contents**Liquidity and Capital Resources**

Since our inception, we have funded operations primarily through the sale of preferred stock and issuance of convertible notes payable. In October 2013, we raised approximately \$68.3 million in net proceeds in our initial public offering. 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. As of September 30, 2013 and December 31, 2012, our principal sources of liquidity were our cash and cash equivalents, which totaled approximately \$4.6 million and \$2.9 million, respectively.

Through September 30, 2013, we have raised net cash proceeds of \$78.6 million through the sales of \$43.8 million of convertible preferred stock and \$34.8 million from the issuance of convertible notes that was subsequently converted into common stock in October 2013 in connection with the closing of our initial public offering.

On December 7, 2012, we authorized the sale of the 2012 Notes, to related parties in the aggregate principal amount of \$15.0 million. In December 2012, we issued \$3.0 million aggregate principal amount of 2012 Notes; in March 2013, we issued \$3.0 million aggregate principal amount of 2012 Notes; in May 2013, we issued \$4.5 million aggregate principal amount of 2012 Notes; and in August 2013, we issued \$4.5 million aggregate principal amount of 2012 Notes. In August 2013, we amended the agreements relating to the 2012 Notes, authorizing the sale of an additional \$3.0 million aggregate principal amount of 2012 Notes and extending the maturity date from September 30, 2013 to December 31, 2013. In September 2013, we issued the additional \$3.0 million aggregate principal amount of 2012 Notes to related parties. The 2012 Notes accrued interest at a rate of 8% per annum. In connection with our initial public offering in October 2013, all outstanding 2012 Notes and interest accrued thereon were converted into shares of our common stock.

On October 30, 2013, we completed our initial public offering and issued 7,728,000 shares of our common stock at an initial offering 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 initial public offering of approximately \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million. We believe that our cash and cash equivalents as of September 30, 2013, together with the net proceeds from our initial public offering will be sufficient to fund our operations through approximately mid-2016. Our ability to continue as a going concern will depend, in large part, on our ability to maintain the necessary capital resources to fund our business and generate positive cash flow from operations, neither of which is certain. The perception of our ability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees.

The following table summarizes our sources and uses of cash:

<i>(In thousands)</i>	Nine Months Ended	
	September 30,	2012
	2013	(unaudited)
Net cash (used in) provided by:		
Operating activities	\$ (11,566)	\$ (11,952)
Investing activities	(28)	(51)
Financing activities	13,288	6

Net increase (decrease) in cash and cash equivalents	\$ 1,694	\$ (11,997)
--	----------	-------------

Table of Contents

During the nine months ended September 30, 2013 and 2012, our operating activities used net cash of \$11.6 million and \$12.0 million, respectively. The use of net cash primarily resulted from our net losses. The decrease in net loss from operations for the nine months ended September 30, 2013 as compared to the nine months ended September 30, 2012 was primarily attributable to \$1.3 million of cash proceeds from the sale of deferred state tax benefits to an unrelated third party. During the nine months ended September 30, 2013 and 2012, our investing activities primarily related to purchases of office furnishings and equipment to facilitate our continued growth. The net cash provided by financing activities during the nine months ended September 30, 2013 was related to \$15.0 million from the aforementioned sale of the convertible notes, offset by \$1.7 million in payments made in preparation for our initial public offering.

Operating Capital Requirements

We expect to incur increasing operating losses for at least the next several years as we commence Phase 3 and Phase 2b clinical trials of our AR-13324 and PG324 product candidates, respectively. We expect that the net proceeds from our initial public offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements through approximately mid-2016 and that we will likely need to raise additional capital thereafter to continue to fund further development of PG324 and our operations.

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 any Phase 3 and Phase 2b clinical trials of our product candidates;

costs of any follow-on development or products;

timing and cost of the ongoing supportive non-clinical 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 expect that we will need to obtain substantial additional funding in order to obtain regulatory approvals on any product candidates and support commercialization and ongoing business activities. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity 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. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay,

We will also incur costs as a public company that we have not previously incurred or previously incurred at lower rates, 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 Securities and Exchange Commission (the SEC) and NASDAQ and various other costs.

Table of Contents

scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that may be less favorable than might otherwise be available.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at September 30, 2013:

	TOTAL	LESS THAN 1 YEAR	1 TO 3 YEARS (in thousands)	3 TO 5 YEARS	MORE THAN 5 YEARS
Operating lease obligations ⁽¹⁾	\$ 964	\$ 310	\$ 414	\$ 240	\$
Convertible notes payable ⁽²⁾	18,000	18,000			
Accrued interest ⁽²⁾	504	504			

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

(2) In connection with our initial public offering, all outstanding notes and accrued interest thereon were converted into common stock.

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.

Jumpstart Our Business Startups Act of 2012

The Jumpstart Our Business Startups Act of 2012 (the JOBS Act) 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 will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Item 3. Quantitative and Qualitative Disclosure 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. We had cash and cash equivalents on hand of \$4.6 million and \$2.9 million as of September 30, 2013 and December 31, 2012, respectively. Given the short-term nature of our cash equivalents, we believe that our interest rate risk is not significant to our financial statements. We do not engage in any hedging activities against changes in interest rates. The 2012 Notes carried a fixed interest rate and, as such, were not subject to interest rate risk. We do not have any foreign currency or other derivative financial instruments.

Item 4. 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 September 30, 2013, the disclosure controls and procedures were effective to provide reasonable assurance that information

Table of Contents

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.

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.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any legal proceedings.

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 AR-13324 and PG324, 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, particularly AR-13324 and PG324, 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 would materially harm our business and we may not be able to earn sufficient revenues and cash flows to continue our operations.

Table of Contents

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. AR-13324 and PG324 are planned to be advanced into Phase 3 and Phase 2b clinical trials, respectively. 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 a new drug application, or 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 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.

Table of Contents

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. The timing for the completion of the studies for our product candidates will require funding beyond the proceeds of our initial public offering. In addition, if regulatory authorities require additional time or studies to assess the safety or efficacy of our product candidates, 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;

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.

Table of Contents

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 the United States, 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 the United States through our own sales force, targeting approximately 10,000 high-prescribing eye-care professionals in the United States. 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 the United States, which will expose us to additional risks of conducting business in international markets.

The non-U.S. markets are an important component of our growth strategy. If we fail to 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:

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;

Table of Contents

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 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 2 or Phase 3 clinical trials that will 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 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.

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 and analyses, 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

Table of Contents

product candidates. 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 upcoming trials for our primary product candidates, AR-13324 and PG324, may not produce the results that we expect. In addition, if based on clinical results of AR-13324 we discontinue the advancement of this product candidate, in certain circumstances we may similarly determine not to advance PG324, which combines AR-13324 with latanoprost. Our clinical trials are also designed to test the use of AR-13324 and PG324 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. AR-13324 showed a 0.1 mmHg change in IOP from day seven to day 28 in our Phase 2b trial, and published clinical data for other ROCK inhibitors similarly have not shown a loss of efficacy over time. However, we have not previously conducted a three-month Phase 2b clinical trial for AR-13324, and therefore there can be no assurance as to the efficacy of AR-13324 beyond 28 days. In addition, our current product candidates remain subject to the risks associated with clinical drug development as indicated above.

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 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;

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 product candidates, our commercial prospects will be adversely impacted and our ability to generate product revenues may be delayed or eliminated.

Table of Contents

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 AR-13324 has been transient hyperemia, or eye-redness. PG324 combines AR-13324 with latanoprost. The main adverse effects of latanoprost include hyperemia, irreversible change in iris color, discoloration of the skin around the eyes and droopiness of eyelids.

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.

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. Sucampo Pharmaceuticals, Inc. recently 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. In addition, early-stage companies that are also developing glaucoma treatments 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;

Table of Contents

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;

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;

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 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

Table of Contents

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 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.

Table of Contents

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 Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, collectively 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, with the first reports due in March 2014 and the reported information to be made publicly available on a searchable website beginning 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.

Table of Contents

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.

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 European Medicines Agency, or EMA, requirements and the requirements of other similar agencies, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practice, or 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 or uses 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.

Table of Contents

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.

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 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 mechanisms of action. 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 development-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;

Table of Contents

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 \$20.9 million, \$11.4 million and \$15.0 million for the nine months ended September 30, 2013 and 2012 and year ended December 31, 2012, respectively. As of September 30, 2013, we had a deficit accumulated during the development stage of \$84.8 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. We have devoted most 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 convertible preferred stock and convertible debt. 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 2 and 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' deficit, financial position, cash flows and working capital.

Table of Contents

We will 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. From the period from inception (June 22, 2005) to September 30, 2013, we have cumulative net cash flows used by operating activities of \$70.4 million. We will need to obtain additional financing to fund our future operations, including completing the development and commercialization of our primary product candidates. We will 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, interest expense 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.

Table of Contents

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.

We believe that our existing cash and cash equivalents, including the net proceeds from our initial public offering will be sufficient to fund our operations through approximately mid-2016. We expect that these funds will not be sufficient to enable us to complete all necessary development or commercially launch our current product candidates. Accordingly, we will 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.

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 development 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.

Table of Contents

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 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. We also do not have any current contractual relationships for the manufacture of commercial supplies of any of our product candidates if and when they are approved. With respect to commercial production of our potential 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. This process is difficult and time consuming and we can give no assurance that we will enter 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

Table of Contents

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.

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 outside of the United States. 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

Table of Contents

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 Good Clinical Practice 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.

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

Table of Contents

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 September 30, 2013, we own two patents in the United States and have 13 patent applications in the United States and certain foreign jurisdictions for our primary product candidates AR-13324 and PG324 (patent protection for PG324 arises from the U.S. patents that cover AR-13324). The two patents individually cover composition of matter and method of use. We own 14 patents and have 20 pending patent applications in the United States and certain foreign jurisdictions relating to our previously discontinued product candidates and other proprietary technology. With respect to our current product candidates, our patents are exclusively in the United States, although we have patent applications pending in the United States and certain foreign jurisdictions. See Business Intellectual Property in the IPO Prospectus 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 U.S. market for our AR-13324 and PG324 product candidates;

we do not at this time own or control a granted European patent or national phase patents in any European jurisdictions that would prevent generic entry into the European market for our AR-13324 product candidate;

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;

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;

Table of Contents

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 abbreviated new drug applications 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 September 30, 2013, we own 16 patents and have 33 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" in the IPO Prospectus for further information about our issued patents and patent applications. Our issued patents include two U.S. patents for composition of matter and method of use covering our lead product candidate, AR-13324 (these patents also cover our other primary product candidate PG324 to the extent that AR-13324 forms a part of PG324). The remainder of our portfolio is made up of patents covering previously discontinued product candidates and pending patent applications that have not yet been issued by the U.S. Patent and Trademark Office, or 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 certain foreign jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and

Table of Contents

other intellectual property protection, especially those relating to life sciences. We do not currently have any issued patents in any foreign jurisdictions that cover our most advanced product candidates. To the extent we are able to obtain patents or other intellectual property rights in any foreign jurisdictions, it may be difficult for us to stop the infringement of our patents or the misappropriation of these intellectual property rights. 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

Table of Contents

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 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

Table of Contents

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.

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.

Any name 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,

Table of Contents

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 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.

Table of Contents

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 23 employees as of September 30, 2013. 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 in the future, we will incur a variety of costs and we may never realize the anticipated benefits of such acquisitions.

We may attempt to acquire 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.

However, if we do undertake any acquisitions, the process of integrating an acquired business, technology, service, products or product candidates 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 could result in additional issuances of equity securities that would dilute the ownership of existing stockholders. Future acquisitions 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. In addition, we may fail to realize the anticipated benefits of any acquisition.

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.

Table of Contents

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;

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

Table of Contents

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 headquarters is located in Bedminster, New Jersey, our research and development facility is located in Research Triangle Park, North Carolina and we have an office in Newport Beach, California. 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 may be highly volatile.

The trading price of our common stock is likely to be volatile. 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:

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 testing and clinical trials;

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 share price may be 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.

Table of Contents

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.

After our initial public offering, our officers and directors, and stockholders who own more than 5% of our outstanding common stock beneficially own approximately 70.9% of our common stock. 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 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. Following our initial public offering, we had 22,205,717 outstanding shares of common stock, of which 15,485,717 shares are subject to lock-up agreements with the underwriters of our initial public offering that restrict the stockholders' ability to transfer shares of our common stock for at least 180 days from the date of the IPO Prospectus. These restrictions are due to expire on April 23, 2014, resulting in these shares becoming eligible for public sale thereafter if they are registered under the Securities Act or if they qualify for an exemption from such registration.

Some of the holders of our common stock are entitled to rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by our affiliates as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading 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 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, 2012, we had net operating losses of approximately \$60.8 million, which may be utilized against future federal and state income taxes. These net operating losses will begin to expire at various dates beginning in 2024, if not utilized. If we

Table of Contents

experience an ownership change for purposes of Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, 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, 2012.

The requirements associated with being a public company will 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 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 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;

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; and

any rules that may be adopted by the Public Company Accounting Oversight Board requiring mandatory audit firm rotation or a supplement to the auditor's report on the financial statements.

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) the last day of the first fiscal year following the fifth anniversary of the completion of our initial public offering; (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.

Table of Contents

Although we are still evaluating the JOBS Act, 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.

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.

Table of Contents

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Unregistered Sales of Equity Securities

In August 2013 and September 2013, we issued \$4.5 million and \$3.0 million, respectively, of convertible notes to related accredited investors. Note 5 to our unaudited financial statements appearing elsewhere in this report further describes the attributes of these notes, including the terms of conversion of these notes. In connection with the completion of our initial public offering in October 2013, all of the convertible notes and accrued interest thereon were converted into shares of our common stock.

In connection with the issuance of the aforementioned convertible notes, in August 2013 and September 2013, we granted warrants to purchase 1,022,727 and 681,816 shares of our convertible preferred stock, respectively. Note 7 to our unaudited financial statements appearing elsewhere in this report further describes the attributes of these warrants, including the terms of exercise of these warrants. Upon the completion of our initial public offering in October 2013, these warrants automatically became exercisable for 340,909 shares of our common stock.

During the three months ended September 30, 2013, we granted stock options to purchase 1,704,752 shares of our common stock to our employees, directors and consultants pursuant to our 2005 Stock Option Plan, as amended, at a weighted-average exercise price of \$3.15 per share. No shares of common stock were issued due to exercises.

We deemed the offers, sales and issuances of the convertible notes and warrants described above to be exempt from registration under the Securities Act, in reliance on Section 4(2) of the Securities Act, including Regulation D and Rule 506 promulgated thereunder, relating to transactions by an issuer not involving a public offering. All purchasers of securities in transactions exempt from registration pursuant to Regulation D represented to us that they were accredited investors and were acquiring the shares for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time. The purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from such registration.

We deemed the grants and exercises of stock options described above to be exempt from registration under the Securities Act in reliance on Rule 701 of the Securities Act as offers and sales of securities under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701. Each of the recipients of securities in any transaction exempt from registration either received or had adequate access, through employment, business or other relationships, to information about us.

All certificates representing the securities issued in the transactions described in this Item 2 included appropriate legends setting forth that the securities had not been offered or sold pursuant to a registration statement and describing the applicable restrictions on transfer of the securities. There were no underwriters employed in connection with any of the transactions set forth above in this Item 2.

Use of Proceeds of Initial Public Offering

On October 30, 2013, we completed our initial public offering and issued 7,728,000 shares of our common stock at an initial offering 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 initial public offering of \$68.3 million, after deducting underwriting discounts and commissions of \$5.4 million and expenses of \$3.6 million. None of the expenses associated with the initial public offering were paid to

directors, officers, persons owning 10% or more of any class of equity securities, or to their associates, or to our affiliates. RBC Capital Markets, LLC and Stifel, Nicolaus & Company, Incorporated acted as joint book-running managers and Canaccord Genuity Inc. and Needham & Company, LLC acted as co-managers for the offering.

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. Shares of our common stock began trading on the NASDAQ Global Market on October 25, 2013. On October 30, 2013, following the sale of 7,728,000 shares of common stock, the offering terminated.

Because the closing of our initial public offering occurred on October 30, 2013, as of September 30, 2013, we had not received the net proceeds from the sale of these securities and therefore had used none of the proceeds by such date. There has been no material change in our planned use of the net proceeds from our initial public offering as described in our final prospectus filed with the SEC on October 28, 2013 pursuant to Rule 424(b).

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

On December 3, 2013, David Mack, MD, resigned from his position as a member of our board of directors, effective immediately.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which is incorporated herein by reference.

Table of Contents

SIGNATURES

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

AERIE PHARMACEUTICALS, INC.

Date: December 5, 2013

/s/ RICHARD J. RUBINO
Richard J. Rubino
Chief Financial Officer
(Principal Financial and Accounting Officer)

Table of Contents

INDEX TO EXHIBITS

EXHIBIT NO.	EXHIBIT
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 By-Laws 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).
10.1	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).
10.2	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).
10.3	Fourth Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan (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).
10.4	Fifth Amendment of Aerie Pharmaceuticals, Inc. 2005 Stock Option Plan (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).
10.5	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).
10.6	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).
10.7	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).
10.8	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).
10.9	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).
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.*
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.*

* Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at September 30, 2013 (unaudited) and December 31, 2012, (ii) Statements of Operations and Comprehensive Loss for the three months ended September 30, 2013 and 2012, the nine months ended September 30, 2013 and 2012 and the period from inception (June 22, 2005) to September 30, 2013 (unaudited), (iii) Statements of Cash Flows for the nine months ended September 30, 2013 and 2012 and for the period from inception (June 22, 2005) to September 30, 2013 (unaudited) and (iv) Notes to Financial Statements (unaudited).

In accordance with Rule 406T of Regulation S-T, the XBRL related information in Exhibit 101 to this Quarterly Report on Form 10-Q is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act, is deemed not filed for purposes of Section 18 of the Exchange Act, and otherwise is not subject to liability under these sections.