Aclaris Therapeutics, Inc.
Form 10-Q
November 18, 2015
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark one)

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

For the quarterly period ended September 30, 2015

OR

o 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 001-37581

Aclaris Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**46-0571712**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)
101 Lindenwood Drive, Suite 400 **Malvern, PA** (Address of principal executive offices)	**19355** (Zip Code)

(484) 324-7933

(Registrant s telephone number, including area code)

N/A

(Former name, former address and former fiscal year, if changed since last report)

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 o No x

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

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 Securities Exchange Act of 1934.

Large accelerated filer o	Accelerated filer o

Non-accelerated filer x (Do not check if a smaller reporting company)

Smaller reporting company o

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Securities Exchange Act of 1934). Yes o No x

The number of outstanding shares of the registrant s common stock, par value $0.00001 per share, as of the close of business on November 17, 2015 was 20,157,503.

Table of Contents

ACLARIS THERAPEUTICS, INC.

INDEX TO FORM 10-Q

Table of Contents

Part I. FINANCIAL INFORMATION

Item 1. Financial Statements

ACLARIS THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(UNAUDITED)

(In thousands, except share and per share data)

	September 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 25,419	$ 10,757
Marketable securities	12,986	5,373
Prepaid expenses and other current assets	955	204
Total current assets	39,360	16,334
Marketable securities		518
Property and equipment, net	761	515
Deferred offering costs	1,943	
Other assets	23	10
Total assets	$ 42,087	$ 17,377
Liabilities, Convertible Preferred Stock and Stockholders Deficit		
Current liabilities:		
Accounts payable	$ 958	$ 1,263
Accrued expenses	939	188
Total current liabilities	1,897	1,451
Deferred rent	3	4
Total liabilities	1,900	1,455
Convertible preferred stock (Series A, B and C), $0.00001 par value; 40,286,041 and 34,090,000 shares authorized at September 30, 2015 and December 31, 2014, respectively; 40,286,041 and 27,341,057 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively; aggregate liquidation preference of $78,282 and $35,882 at September 30, 2015 and December 31, 2014, respectively	78,305	36,677
Stockholders deficit:		
Common stock, $0.00001 par value; 110,000,000 and 77,000,000 shares authorized at September 30, 2015 and December 31, 2014, respectively; 2,730,427 and 2,730,427 shares issued and outstanding		
Additional paid-in capital		
Accumulated other comprehensive income (loss)	1	(6)
Accumulated deficit	(38,119)	(20,749)
Total stockholders deficit	(38,118)	(20,755)

Total liabilities, convertible preferred stock and stockholders deficit	$ 42,087	$ 17,377

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONDENSED CONSOLIDATED 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,	
	2015	2014	2015	2014
Revenue	$	$	$	$
Operating expenses:				
Research and development	9,408	2,097	12,937	4,453
General and administrative	1,232	565	2,928	1,478
Total operating expenses	10,640	2,662	15,865	5,931
Loss from operations	(10,640)	(2,662)	(15,865)	(5,931)
Interest income	7	5	16	11
Net loss	(10,633)	(2,657)	(15,849)	(5,920)
Accretion of convertible preferred stock	(995)	(479)	(2,353)	(1,394)
Net loss attributable to common stockholders	$ (11,628)	$ (3,136)	$ (18,202)	$ (7,314)
Net loss per share attributable to common stockholders, basic and diluted	$ (5.11)	$ (1.75)	$ (8.44)	$ (4.36)
Weighted average common shares outstanding, basic and diluted	2,274,617	1,794,909	2,155,685	1,675,976
Other comprehensive income (loss):				
Unrealized gain (loss) on marketable securities, net of tax of $0	1	(3)	7	(3)
Total other comprehensive income (loss)	1	(3)	7	(3)
Comprehensive loss	$ (10,632)	$ (2,660)	$ (15,842)	$ (5,923)

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK

AND STOCKHOLDERS DEFICIT (UNAUDITED)
FOR THE PERIOD FROM JANUARY 1, 2015 TO SEPTEMBER 30, 2015

(In thousands, except share data)

	Series A, B and C Convertible Preferred Stock		Common Stock					
	Shares	Amount	Shares	Par Value	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Deficit
Balance at January 1, 2015	27,341,057	$ 36,677	2,730,427	$	$	$ (6)	$ (20,749)	$ (20,755)
Issuance of Series C convertible preferred stock, net of issuance costs of $136	12,944,984	39,864						
Unrealized gain on marketable securities						7		7
Stock-based compensation expense					243			243
Accretion of convertible preferred stock to redemption value		1,764			(243)		(1,521)	(1,764)
Net loss							(15,849)	(15,849)
Balance at September 30, 2015	40,286,041	$ 78,305	2,730,427	$	$	$ 1	$ (38,119)	$ (38,118)

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)

(In thousands)

	Nine Months Ended September 30,	
	2015	**2014**
Cash flows from operating activities:		
Net loss	$ (15,849)	$ (5,920)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	57	9
Stock-based compensation expense	243	8
Deferred rent	(1)	1
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(764)	(397)
Accounts payable	(491)	920
Accrued expenses	573	505
Net cash used in operating activities	(16,232)	(4,874)
Cash flows from investing activities:		
Purchases of property and equipment	(375)	(148)
Purchases of marketable securities	(13,002)	
Proceeds from sales and maturities of marketable securities	5,914	3,734
Net cash (used in) provided by investing activities	(7,463)	3,586
Cash flows from financing activities:		
Proceeds from issuance of convertible preferred stock, net of issuance costs	39,864	10,588
Payments of initial public offering costs	(1,507)	
Net cash provided by financing activities	38,357	10,588
Net increase in cash and cash equivalents	14,662	9,300
Cash and cash equivalents at beginning of period	10,757	9,588
Cash and cash equivalents at end of period	$ 25,419	$ 18,888
Supplemental disclosure of non-cash investing and financing activities:		
Additions to property and equipment purchases included in accounts payable	$ 19	$ 8
Accretion of convertible preferred stock to redemption value	$ 1,764	$ 1,393
Fair value of preferred stock purchased put option on date of issuance	$	$ 1,039
Deferred offering costs included in accounts payable and accrued expenses	$ 436	$

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

September 30, 2015

(Amounts in thousands, except share and per share data)

1. Organization and Nature of Business

Aclaris Therapeutics, Inc. (the Company) was incorporated under the laws of the State of Delaware in 2012. The Company is a clinical-stage specialty pharmaceutical company focused on identifying, developing and commercializing innovative and differentiated drugs to address significant unmet needs in dermatology. The Company s lead drug candidate, A-101, is a proprietary high-concentration hydrogen peroxide topical solution that the Company is developing as a prescription treatment for seborrheic keratosis (SK), a common non-malignant skin tumor. The Company has completed three Phase 2 clinical trials of A-101 in patients with SK.

On July 17, 2015, Aclaris Therapeutics International Limited was established as a wholly-owned subsidiary of the Company.

Initial Public Offering

On October 6, 2015, the Company s registration statement on Form S-1 relating to its initial public offering of its common stock (the IPO) was declared effective by the Securities and Exchange Commission (SEC). The shares began trading on The NASDAQ Global Select Market on October 7, 2015. The IPO closed on October 13, 2015, and 5,000,000 shares of common stock were sold at a price to the public of $11.00 per share, for aggregate gross proceeds of $55,000. In addition, upon the closing of the IPO, all of the Company s outstanding convertible preferred stock was converted into an aggregate total of 11,677,076 shares of common stock.

On October 12, 2015, the underwriters of the IPO exercised in full their option to purchase additional shares, and on October 13, 2015, the Company s sold 750,000 additional shares of common stock at a price to the public of $11.00 per share, for aggregate gross proceeds of $8,250.

The Company paid to the underwriters underwriting discounts and commissions of $4,428 in connection with the IPO, including the underwriters exercise of their option to purchase additional shares. In addition, the Company incurred expenses of approximately $2,200 in connection with the IPO. Thus, the net offering proceeds to the Company, after deducting underwriting discounts and commissions and offering

expenses, were $56,623.

Reverse Stock Split

On September 24, 2015, the Company effected a 1-for-3.45 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company s convertible preferred stock (see Note 6). Accordingly, all share and per share amounts for all periods presented in these financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios.

Liquidity

The Company s financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. At September 30, 2015, the Company had working capital of $37,463, an accumulated deficit of $38,119 and cash, cash equivalents and marketable securities of $38,405. Upon consummation of its IPO on October 13, 2015, the Company received cash proceeds, net of underwriting discounts and commissions, of $58,823. The Company has not generated any product revenues and has not achieved profitable operations. There is no assurance that profitable operations will ever be achieved, and, if achieved, will be sustained on a continuing basis. In addition, development activities, clinical and pre-clinical testing, and commercialization of the Company s products will require significant additional financing.

The Company expects that its cash and cash equivalents as of September 30, 2015, together with the proceeds received in connection with its IPO in October 2015, will be sufficient to fund its operations for at least the next 24 months. The future

Table of Contents

viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company s failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). The financial statements include the consolidated accounts of the Company and its wholly-owned subsidiary, Aclaris Therapeutics International Limited. All intercompany transactions have been eliminated.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, the accrual of research and development expenses and the valuation of common stock and stock-based awards.

Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from the Company s estimates.

Unaudited Interim Financial Information

The accompanying condensed consolidated balance sheet as of September 30, 2015, the condensed consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2015 and 2014, the condensed consolidated statement of convertible preferred stock and stockholders deficit for the nine months ended September 30, 2015, and the condensed consolidated statements of cash flows for the nine months ended September 30, 2015 and 2014 are unaudited. The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company s financial position as of September 30, 2015, the results of its operations and comprehensive loss for the three and nine months ended September 30, 2015 and 2014 and its cash flows for the nine months ended September 30, 2015 and 2014. The financial data and other information disclosed in these notes related to the nine months ended September 30, 2015 and 2014 are unaudited. The results for the nine months ended September 30, 2015 are not necessarily indicative of results to be expected for the year ending December 31, 2015, any other interim periods, or any future year or period. The unaudited interim financial statements of the Company included herein have been prepared, pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. These unaudited condensed consolidated financial statements should be read in conjunction

with the audited financial statements and the notes thereto for the year ended December 31, 2014 included in the Company s final prospectus filed on October 8, 2015 with the SEC pursuant to Rule 424(b)(4) of the Securities Act of 1933, as amended (the Securities Act).

Significant Accounting Policies

The Company s significant accounting policies are disclosed in the audited financial statements for the year ended December 31, 2014 included in the Company s final prospectus filed on October 8, 2015 with the SEC pursuant to the Securities Act. Since the date of such financial statements, there have been no changes to the Company s significant accounting policies, other than those detailed below.

Deferred Offering Costs

The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs (non-current) until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders deficit as a reduction of additional paid-in capital generated as a result of the offering. As of September 30, 2015, the Company had recorded $1,943 of deferred offering costs incurred in

Table of Contents

connection with the IPO. The deferred offering costs were offset against the net proceeds received upon completion of the IPO in October 2015.

3. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company s assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

	Fair Value Measurements as of September 30, 2015 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 25,020	$	$	$ 25,020
Marketable securities		12,986		12,986
	$ 25,020	$ 12,986	$	$ 38,006

	Fair Value Measurements as of December 31, 2014 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 10,012	$	$	$ 10,012
Marketable securities		5,891		5,891
	$ 10,012	$ 5,891	$	$ 15,903

As of September 30, 2015 and December 31, 2014, the Company s cash equivalents, which were invested in money market funds, were valued based on Level 1 inputs. In determining the fair value of its corporate debt securities and U.S. government agency debt securities as of September 30, 2015 and December 31, 2014, the Company relied on quoted prices for identical securities in markets that are not active, a Level 2 input. These quoted prices were obtained by the Company with the assistance of a third-party pricing service based on available trade, bid and other observable market data for identical securities. Quarterly, the Company compares the quoted prices obtained from the third-party pricing service to other available independent pricing information to validate the reasonableness of the quoted prices provided. The Company evaluates whether adjustments to third-party pricing is necessary and, historically, the Company has not made adjustments to quoted prices obtained from the third-party pricing service. During the nine months ended September 30, 2015 and the years ended December 31, 2014, there were no transfers between Level 1, Level 2 and Level 3.

As of September 30, 2015 and December 31, 2014, the fair value of the Company s available-for-sale marketable securities by type of security was as follows:

	September 30, 2015			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Marketable securities:				

Corporate debt securities	$ 12,985	$ 1	$	$ 12,986
	$ 12,985	$ 1	$	$ 12,986

	December 31, 2014			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Marketable securities:				
Corporate debt securities	$ 5,096	$	$ (6)	$ 5,090
U.S. government agency debt securities	801			801
	$ 5,897	$	$ (6)	$ 5,891

Table of Contents

As of September 30, 2015 and December 31, 2014, the Company s corporate debt securities had credit ratings of A and above and remaining maturities of less than 8 months and less than 13 months, respectively.

4. Property and Equipment, Net

Property and equipment, net consisted of the following:

	September 30, 2015	December 31, 2014
Computer equipment	$ 190	$ 36
Manufacturing equipment	578	
Construction in progress	77	506
	845	542
Less: Accumulated depreciation	(84)	(27)
	$ 761	$ 515

Depreciation expense was $32 and $3 for the three months ended September 30, 2015 and 2014, respectively, and $57 and $9 for the nine months ended September 30, 2015 and 2014, respectively. Construction in progress as of September 30, 2015 consisted of manufacturing equipment, which is expected to be placed into service in 2016.

5. Accrued Expenses

Accrued expenses consisted of the following:

	September 30, 2015	December 31, 2014
Payroll and payroll-related costs	$ 463	$
Clinical trial expenses	284	163
Deferred offering costs	178	
Other	14	25
	$ 939	$ 188

6. Convertible Preferred Stock

The Company issued Series A, Series B and Series C convertible preferred stock (collectively, the Preferred Stock). As of September 30, 2015 and December 31, 2014, the Company s certificate of incorporation, as amended and restated, authorized the Company to issue 40,286,041 shares and 34,090,000 shares, respectively, of Preferred Stock, $0.00001 par value.

In September 2014, the Company entered into a stock purchase agreement pursuant to which the Company agreed to sell to the investors an initial issuance (the First Tranche) of 6,451,057 shares of Series B convertible preferred stock (the Series B preferred stock) at $1.65 per share for gross proceeds of $10,644. Per the terms of that stock purchase agreement, upon the successful attainment of two specified milestones, the Company was entitled to call a second tranche of 6,451,057 shares of Series B preferred stock at $1.65 per share (the purchased put option). The Company had the right, but not the obligation, to exercise its purchased put option after successful attainment of the specified milestones as confirmed by a vote of five-sixths of the members of the Company s board of directors and 60% of voting stockholders of the Company. The two milestones related to (i) the successful achievement of the primary efficacy endpoint and demonstrated safety of a specified Phase 2b clinical trial of A-101 in patients with SK, and (ii) the occurrence of an end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA), as a result of which the FDA did not raise any objection to the Company proceeding to a Phase 3 clinical trial of A-101 in patients with SK.

In connection with the initial issuance of Series B preferred stock in September 2014, the Company recorded the First Tranche transaction, net of issuance costs of $60, and the $1,039 issuance-date fair value of the purchased put option. The purchased put option was recorded as a charge to accumulated deficit within stockholders deficit and as an increase to the carrying value of Series B preferred stock based on the Company s conclusion that the purchased put option met the equity classification criteria at time of issuance as the purchased put option (i) was a freestanding financial instrument that did not

Table of Contents

require the Company to issue shares that are potentially redeemable and (ii) required gross physical settlement in all circumstances.

The fair value of the purchased put option was determined on the date of its issuance using the Black-Scholes option-pricing model with the following assumptions and inputs: risk-free interest rate of 0.08%, expected term of nine months, expected volatility of 80.0%, no expected dividends and fair value of underlying instruments of $1.65. The fair value calculation also included an estimate of a 60% probability of occurrence of the successful attainment of the specified milestones that triggered the Company s ability to exercise the purchased put option, as well as an estimate of a 60% probability of the Company exercising the purchased put option, if it became exercisable.

On August 28, 2015, the Company issued 12,944,984 shares of Series C convertible preferred stock (Series C preferred stock) at a price of $3.09 per share for gross proceeds of $40,000. The rights and preferences of the Series C preferred stock were similar to those of the Series A and Series B preferred stock, except that (1) the Original Issue Price for Series C preferred stock was $3.09 per share, (2) the holders of the Series C preferred stock did not have redemption rights, and (3) the holders of the Series C preferred stock had specified protective rights not held by the holders of the Series A and Series B preferred stock.

In connection with the closing of the Series C preferred stock financing, the redemption rights of the Series A and Series B preferred stock were removed at that time. As a result of the removal of the redemption rights, as of August 25, 2015, the Company ceased the periodic recording of adjustments to accrete the carrying values of Series A and Series B preferred stock to their respective redemption values through September 30, 2019, which had been the first required redemption date. The accretion for the three and nine months ended September 30, 2015 represents both the issuance costs and cumulative accrued but unpaid dividends through August 28, 2015. Subsequent to August 28, 2015, the Company was no longer required to record the accumulated undeclared dividends on its balance sheet, but is required to deduct accumulated undeclared dividends as part of its loss per share calculation.

Also in connection with the closing of the Series C preferred stock financing, the terms of a qualified public offering requiring the conversion of all shares of the Company s Preferred Stock into common stock were changed and the Series B preferred stock purchase agreement was amended to terminate the Company s purchased put option with respect to a second tranche of Series B preferred stock.

The Preferred Stock was subject to redemption under certain deemed liquidation events, and as such, the Preferred Stock was considered contingently redeemable for accounting purposes. Accordingly, the Preferred Stock was recorded within temporary equity in the condensed consolidated financial statements. The Company has not adjusted the Preferred Stock to its redemption amount at each reporting period, as the redemption of such Preferred Stock was not deemed probable of occurrence during the periods presented. The redemption of the Preferred Stock was not considered probable as the redemption was contingent on the occurrence of such deemed liquidation events which the Company concluded were not probable for the periods presented.

Preferred Stock consisted of the following:

	September 30, 2015			
Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion

Series A convertible preferred stock	20,890,000	20,890,000	$ 26,178	$ 26,491	6,055,060
Series B convertible preferred stock	6,451,057	6,451,057	12,263	11,496	1,869,859
Series C convertible preferred stock	12,944,984	12,944,984	39,864	40,295	3,752,157
	40,286,041	40,286,041	$ 78,305	$ 78,282	11,677,076

	December 31, 2014				
	Preferred Shares Authorized	**Preferred Shares Issued and Outstanding**	**Carrying Value**	**Liquidation Preference**	**Common Stock Issuable Upon Conversion**
Series A convertible preferred stock	20,890,000	20,890,000	$ 24,879	$ 25,023	6,055,060
Series B convertible preferred stock	13,200,000	6,451,057	11,798	10,859	1,869,859
	34,090,000	27,341,057	$ 36,677	$ 35,882	7,924,919

Table of Contents

Each share of Preferred Stock was convertible into common stock at the option of the stockholder at any time after the date of issuance. Each share of the Preferred Stock was convertible into shares of common stock, at the applicable conversion ratio of each series of Preferred Stock then in effect, upon the earlier of (i) a qualified public offering with net proceeds of not less than $40,000 and a price of not less than $12.80 per share, subject to appropriate adjustment for any stock dividend, stock split, combination or other similar recapitalization, and (ii) the date specified by written consent or agreement of the holders of 60% of the then-outstanding shares of Preferred Stock, voting together as a single class (on an as-converted basis).

The conversion ratio of each series of Preferred Stock was determined by dividing the Original Issue Price of each series of preferred stock by the Conversion Price of each series. The Conversion Price was $3.45 for Series A preferred stock, $5.6925 for Series B preferred stock and $10.6605 for Series C preferred stock and was subject to adjustment as set forth in the Company s certificate of incorporation, as amended and restated. As of September 30, 2015 and December 31, 2014, all outstanding shares of Series A and Series B preferred stock were convertible into common stock on a 3.45-for-1 basis.

As previously discussed in Note 1, the Company completed its IPO in October 2015. Upon the closing of the IPO, all of the Company s outstanding Preferred Stock was converted into an aggregate total of 11,677,076 shares of common stock.

7. Stockholders Deficit

Common Stock

As of September 30, 2015 and December 31, 2014, the Company s certificate of incorporation, as amended and restated, authorized the Company to issue 110,000,000 shares and 77,000,000 shares, respectively, of $0.00001 par value common stock.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company s stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any, subject to the preferential dividend rights of the Preferred Stock. When dividends are declared on shares of common stock, the Company must declare at the same time a dividend payable to the holders of Preferred Stock equivalent to the dividend amount they would receive if each preferred share were converted into common stock. The Company may not pay dividends to common stockholders until all dividends accrued or declared but unpaid on the Preferred Stock have been paid in full. No dividends had been declared through September 30, 2015.

As of September 30, 2015 and December 31, 2014, the Company had reserved 13,216,245 shares and 8,425,181 shares of common stock, respectively, for the conversion of the outstanding shares of Series A, Series B and Series C preferred stock (see Note 6) and the exercise of outstanding stock options and the number of shares remaining available for future grant under the Company s 2012 Plan (see Note 8).

Restricted Common Stock

The Company has granted restricted common stock with time-based vesting conditions. Unvested shares of restricted common stock may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. Upon a qualified public offering or a change in control of the Company, all unvested shares of restricted common stock vest immediately.

In July 2012, the Company issued 2,730,427 shares of common stock to its founders in connection with the Company s formation, of which 1,918,834 shares were subject to vesting pursuant to restricted stock agreements, with 25% of such shares vesting in July 2013 and the remaining 75% vesting in equal monthly installments over a three-year period thereafter. The estimated grant-date fair value of these restricted common shares was $0.00001 per share, equal to the par value of each share. As of September 30, 2015 and December 31, 2014, 399,757 shares and 759,538 shares, respectively, were subject to repurchase.

The table below summarizes the Company s restricted stock activity since January 1, 2015:

Table of Contents

	Number of Shares		Weighted Average Grant Date Fair Value Per Share
Unvested restricted common stock as of December 31, 2014	759,538	$	0.00001
Vested	359,781	$	0.00001
Unvested restricted common stock as of September 30, 2015	399,757	$	0.00001

The aggregate intrinsic value of restricted stock awards that vested during the three months ended September 30, 2015 and 2014 and the nine months ended September 30, 2015 and 2014 was $1,091, $170, $2,026 and $269, respectively.

As previously discussed in Note 1, the Company completed its IPO in October 2015. As a result of the IPO, all of the Company s remaining unvested restricted common stock vested in full.

8. Stock-Based Awards

2012 Equity Compensation Plan

The Company s 2012 Equity Compensation Plan, as amended and restated (the 2012 Plan), provides for the Company to sell or issue common stock or restricted common stock, or to grant incentive stock options or nonqualified stock options for the purchase of common stock, to employees, members of the board of directors and consultants of the Company. The 2012 Plan is administered by the board of directors or, at the discretion of the board of directors, by a committee of the board. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or their committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of stock options may not be greater than ten years. The Company generally grants stock-based awards with service conditions only (service-based awards).

Stock options granted under the 2012 Plan generally vest over four years and expire after ten years.

The total number of shares of common stock that may be issued under the 2012 Plan was 500,262 shares as of December 31, 2014. On August 25, 2015, the Company effected an increase in the number of shares of common stock reserved for issuance under the 2012 Plan from 500,262 shares to 1,539,169 shares.

As of September 30, 2015 and December 31, 2014, 398,645 and 0 shares remained available for grant under the 2012 Plan, respectively.

As required by the 2012 Plan, the exercise price for stock options granted is not to be less than the fair value of common shares as determined by the Company as of the date of grant. The Company values its common stock by taking into consideration its most recently available valuation of

common shares performed by management and the board of directors as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant.

2015 Equity Incentive Plan

On September 15, 2015, the Company s board of directors adopted and on September 16, 2015, the Company s stockholders approved the 2015 Equity Incentive Plan (the 2015 Plan), which became effective in connection with the IPO in October 2015. As of the time the 2015 Plan became effective, no further grants may be made under the 2012 Plan. The 2015 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance stock awards, cash-based awards and other stock-based awards. The number of shares initially reserved for issuance under the 2015 Plan was 2,784,395 shares of common stock. The number of shares of common stock that may be issued under the 2015 Plan will automatically increase on January 1 of each year, beginning on January 1, 2016 and ending on January 1, 2025, in an amount equal to the lesser of (i) 4.0% of the shares of the Company s common stock outstanding on December 31 of the preceding calendar year or (ii) an amount determined by the Company s board of directors. The shares of common stock underlying any awards that expire, are otherwise terminated, settled in cash or repurchased by the Company under the 2015 Plan and the 2012 Plan will be added back to the shares of common stock available for issuance under the 2015 Plan. On October 6, 2015, in conjunction with the pricing of the IPO, the Company issued 89,800 stock options.

Table of Contents

Stock Option Valuation

The assumptions that the Company used to determine the fair value of the stock options granted to employees and directors were as follows, presented on a weighted average basis:

	Nine Months Ended September 30, 2015	Nine Months Ended September 30, 2014
Risk-free interest rate	1.73%	1.85%
Expected term (in years)	6.22	6.18
Expected volatility	96.65%	116.05%
Expected dividend yield	0%	0%

The Company recognizes compensation expense for only the portion of awards that are expected to vest. For the three and nine months ended September 30, 2015 and 2014, the Company applied an expected forfeiture rate of 0%.

Stock Options

The following table summarizes stock option activity under the 2012 Plan from January 1, 2015 through September 30, 2015:

	Number of Shares		Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)		Aggregate Intrinsic Value
Outstanding as of December 31, 2014	500,262	$	1.22	9.77	$	305
Granted	640,262	$	10.66			
Exercised						
Forfeited and canceled						
Outstanding as of September 30, 2015	1,140,524	$	6.79	9.53	$	5,110
Options vested and expected to vest as of September 30, 2015	1,140,524	$	6.79	9.53	$	5,110
Options exercisable as of September 30, 2015	130,075(1)	$	0.67	8.78	$	1,343

(1) All options granted to date under the 2012 Plan are exercisable immediately, subject to a repurchase right in the Company s favor that lapses as the option vests. This amount reflects the number of shares under options that were vested, as opposed to exercisable, as of September 30, 2015.

The weighted average grant-date fair value of stock options granted during the nine months ended September 30, 2015 was $8.34 per share.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company s common stock for those stock options that had exercise prices lower than the fair value of the Company s common stock.

Stock-Based Compensation

For the three and nine months ended September 30, 2015 and 2014, the Company recorded stock-based compensation in the following expense categories of its statements of operations and comprehensive loss:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	**2014**	**2015**	**2014**
Research and development	$ 47	$ 2	$ 74	$ 4
General and administrative	111	4	169	4
	$ 158	$ 6	$ 243	$ 8

Table of Contents

As of September 30, 2015, the Company had an aggregate of $5,906 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 3.53 years.

9. Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Numerator:				
Net loss	$ (10,633)	$ (2,657)	$ (15,849)	$ (5,920)
Accretion of convertible preferred stock to redemption value	(995)	(479)	(2,353)	(1,394)
Net loss attributable to common stockholders	$ (11,628)	$ (3,136)	$ (18,202	$ (7,314)
Denominator:				
Weighted average shares of common stock outstanding	2,730,427	2,730,427	2,730,427	2,730,427
Less: Weighted average shares of unvested restricted common stock outstanding	(455,810)	(935,518)	(574,742)	(1,054,451)
Weighted average common shares outstanding used in calculating net loss per share attributable to common stockholders, basic and diluted	2,274,617	1,794,909	2,155,685	1,675,976
Net loss per share attributable to common stockholders, basic and diluted	$ (5.11)	$ (1.75)	$ (8.44)	$ (4.36)

To calculate net loss attributable to common stockholders for the period, the Company reduced the net loss for the accretion of issuance costs and cumulative dividends accrued but not paid through August 28, 2015, and the remaining cumulative dividends accrued but not paid through September 30, 2015.

The Company s potential dilutive securities, which include stock options, unvested restricted common stock and Preferred Stock, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The following potential common shares, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to common stockholders for both the three and nine months ended September 30, 2015 and 2014 because including them would have had an anti-dilutive effect:

	2015	2014
Stock options to purchase common stock	1,140,524	168,110
Unvested restricted common stock	399,757	879,466

Convertible preferred stock (as converted to common stock)	11,677,076	7,924,919
	13,217,357	8,972,495

10. Commitments and Contingencies

Lease

In September 2013, the Company entered into a sublease agreement for its office space with related parties (see Note 11), with a term ending on November 30, 2016. As part of an amendment to the sublease agreement entered into in December 2014, the Company increased the amount of office space to be leased and agreed to new monthly lease terms commencing in January 2015. On August 14, 2015, the Company further amended its sublease agreement to increase the square footage of the space and to extend the term of the lease to November 2019. Rent expense under operating leases was $34 and $17 for the three months ended September 30, 2015 and 2014, respectively, and $86 and $50 for the nine months

Table of Contents

ended September 30, 2015 and 2014, respectively. The Company recognizes rent expense on a straight-line basis over the lease period and has accrued for rent expense incurred but not yet paid.

As of September 30, 2015, future minimum lease payments under the sublease were as follows:

Years Ending December 31,		
2015	$	42
2016		193
2017		198
2018		202
2019		189
Total	$	824

11. Related Party Transactions

In September 2013, the Company entered into a direct sublease agreement with NeXeption, Inc. (NeXeption) for its leased space (see Note 10). A member of the Company s board of directors is a current executive officer of NeXeption. Total payments made during the three months ended September 30, 2015 and 2014 and the nine months ended September 30, 2015 and 2014 under these sublease agreements were $34, $17, $86 and $50, respectively.

In February 2014, the Company entered into a services agreement with NST, LLC (NST) under which NST provides certain pharmaceutical development, management and other administrative services to the Company. Certain officers of the Company are also founding partners of NST. Under the same agreement, the Company also provides services to NST and is reimbursed for those services. The Company may offset any payments owed by the Company to NST against payments that are owed by NST to the Company for the provision of NST personnel, including consultants, to the Company. During the three months ended September 30, 2015 and 2014 and nine months ended September 30, 2015 and 2014, gross expenses incurred by the Company under the services agreement totaled $105, $119, $358 and $358, respectively, and gross expenses charged to NST by the Company totaled $132, $103, $376 and $310, respectively. For the three months ended September 30, 2015 and 2014 and nine months ended September 30, 2015 and 2014, the Company recorded $36, $79, $172 and $238, respectively, of general and administrative expenses and $63, $63, $190 and $190, respectively, as a reduction of research and development expenses related to these transactions. During the three months ended September 30, 2015 and 2014 and nine months ended September 30, 2015 and 2014, payments made to NST by the Company totaled $0, $5, $16 and $37, respectively. Related to this agreement, no amounts were due to or due from NST at September 30, 2015.

12. License Agreement with Rigel Pharmaceuticals, Inc.

In August 2015, the Company entered into an exclusive, worldwide license and collaboration agreement with Rigel Pharmaceuticals, Inc. (Rigel) for the development and commercialization of products containing specified JAK inhibitors developed by Rigel. Under this agreement, the Company intends to develop these JAK inhibitors for the treatment of alopecia areata and potentially for other dermatological conditions. During the three months ended September 30, 2015, the Company made an upfront non-refundable payment of $8,000 to Rigel. In addition, the Company has agreed to make aggregate payments of up to $80,000 upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals. Further, the Company has agreed to pay up to an additional $10,000 to Rigel upon the achievement of

a second set of development milestones. With respect to any products the Company commercializes under the agreement, the Company will pay Rigel quarterly tiered royalties on its annual net sales of each product at a high single-digit percentage of annual net sales, subject to specified reductions, until the date that all of the patent rights for that product have expired, as determined on a country-by-country and product-by-product basis or, in specified countries under specified circumstances, ten years from the first commercial sale of such product.

The agreement terminates on the date of expiration of all royalty obligations unless earlier terminated by either party for a material breach. The Company may also terminate the agreement without cause at any time upon advance written notice to Rigel. Rigel, after consultation with the Company, will be responsible for maintaining and prosecuting the patent rights, and the Company will have final decision-making authority regarding such patent rights for a product in the United States and the European Union. To the extent that the Company and Rigel jointly develop intellectual property, the parties will confer and decide which party will be responsible for filing, prosecuting and maintaining those patent rights. The agreement also establishes a joint steering committee composed of an equal number of representatives for each party which will monitor progress in the development of products.

Table of Contents

The Company accounted for the transaction as an asset acquisition as the licensing arrangement did not meet the definition of a business pursuant to the guidance prescribed in Accounting Standards Codification Topic 805, *Business Combinations*. Accordingly, the Company recorded the $8,000 upfront payment as research and development expense in the three months ended September 30, 2015. The Company will record as expense any contingent milestone payments or royalties in the period in which such liabilities are incurred. The Company concluded that licensing arrangement with Rigel did not meet the definition of a business because the transaction principally resulted in its acquisition of intellectual property. As part of the transaction, the Company did not acquire any employees or tangible assets, or any processes, protocols or operating systems. In addition, at the time of the acquisition, there were no activities being conducted related to the licensed patents. The Company will expense the acquired intellectual property asset as of the acquisition date on the basis that costs of intangible assets that are purchased from others for use in research and development activities and that have no alternative future uses are research and development costs at the time the costs are incurred.

13. Income Taxes

The Company did not record a federal or state income tax benefit for the Company s losses for the three and nine months ended September 30, 2015 and 2014 due to the Company s conclusion that a valuation allowance is required.

14. Subsequent Events

On October 6, 2015, in connection with the IPO, the Company s Registration Statement on Form S-1 was declared effective by the SEC, and on October 7, 2015, the Company s Common Stock began trading on the NASDAQ Global Select Market under the symbol ACRS.

On October 13, 2015, immediately prior to the closing of the IPO, all outstanding shares of Preferred Stock automatically converted into shares of Common Stock at the applicable conversion ratio then in effect. As a result of the conversion, as of October 13, 2015, the Company had no shares of Preferred Stock outstanding.

On October 13, 2015, the Company closed the IPO. The Company received net proceeds of $56,623 from the IPO, net of underwriting discounts and commissions and other estimated offering expenses (see Note 1).

On October 13, 2015, the Board granted 89,800 options with an exercise price of $11.00 per share pursuant to the 2015 Plan. The awards vest over periods of between three and four years and expire after ten years (see Note 8).

On October 13, 2015, in connection with the IPO, the Company amended and restated its certificate of incorporation to reflect the following authorized share increases: 110,000,000 shares of capital stock, consisting of 100,000,000 shares of common stock, par value $0.00001 per share and 10,000,000 shares of preferred stock, par value $0.00001 per share.

17

Table of Contents

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

Certain statements contained in this Quarterly Report on Form 10-Q may constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words or phrases would be, will allow, intends to, will likely result, are expected to, will continue, is anticipated, estimate, project, or similar expressions, or the negative of such words or phrases, are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events. Because such statements include risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements due to a number of factors, including risks related to:

- *our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;*

- *the success and timing of our preclinical studies and clinical trials and regulatory approval of protocols for future clinical trials;*

- *the difficulties in obtaining and maintaining regulatory approval of our product candidates, and the labeling under any approval we may obtain;*

- *our plans and ability to develop, manufacture and commercialize our product candidates;*

- *our failure to recruit or retain key scientific or management personnel or to retain our executive officers;*

- *the size and growth of the potential markets for our product candidates and our ability to serve those markets;*

- *regulatory developments in the United States and foreign countries;*

- *the rate and degree of market acceptance of any of our product candidates;*

- *obtaining and maintaining intellectual property protection for our product candidates and our proprietary technology;*

- *the successful development of our commercialization capabilities, including sales and marketing capabilities;*

- *recently enacted and future legislation and regulation regarding the healthcare system;*

- *the success of competing therapies and products that are or become available; and*

- *the performance of third parties, including contract research organizations and third-party manufacturers.*

These and other factors that could cause or contribute to these differences are described in this Quarterly Report on Form 10-Q in Part II Item 1A, Risk Factors, and under similar captions in our other filings with the Securities and Exchange Commission. Statements made herein are as of the date of the filing of this Form 10-Q with the Securities and Exchange Commission and should not be relied upon as of any subsequent date. Unless otherwise required by applicable law, we do not undertake, and we specifically disclaim, any obligation to update any forward-looking statements to reflect occurrences, developments, unanticipated events or circumstances after the date of such statement.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes that appear in 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, 2014, which are included in our final prospectus filed with the Securities and Exchange Commission, or SEC, on October 8, 2015 pursuant to Rule 424(b) of the Securities Act of 1933, as amended, or the Securities Act.

Overview

We are a clinical-stage specialty pharmaceutical company focused on identifying, developing and commercializing innovative and differentiated drugs to address significant unmet needs in dermatology. Our lead drug candidate, A-101, is a proprietary high-concentration hydrogen peroxide topical solution that we are developing as a prescription treatment for seborrheic keratosis, or SK, a common non-malignant skin tumor. We have completed three Phase 2 clinical trials of A-101 in over 300 patients with SK. In these trials, following one or two applications of A-101, we observed clinically relevant and

Table of Contents

statistically significant improvements in clearing SK lesions on the face, trunk and extremities of the body. We plan to commence three Phase 3 clinical trials of A-101 in patients with SK in the first quarter of 2016 and, if the results of these trials are favorable, to submit a New Drug Application, or NDA, for A-101 for the treatment of SK to the U.S. Food and Drug Administration, or FDA, in the fourth quarter of 2016. We also intend to develop A-101 as a prescription treatment for common warts and A-102, a proprietary gel dosage form of hydrogen peroxide, as a prescription treatment for SK and common warts. We recently in-licensed the exclusive, worldwide rights to inhibitors of the Janus kinase, or JAK, family of enzymes, for specified dermatological conditions. We plan to develop these JAK inhibitors, A-201 and A-301, as potential treatments for hair loss associated with an autoimmune skin disease known as alopecia areata, or AA, and potentially for other dermatological conditions. We intend to in-license or acquire additional drug candidates for other dermatological conditions to build a fully integrated dermatology company.

Since our inception in July 2012, we have devoted substantially all of our resources to organizing and staffing our company, business planning, raising capital, developing A-101 for the treatment of SK, building our intellectual property portfolio, developing our supply chain and engaging in other discovery and clinical activities in dermatology. Through the date of this report, we have not generated any revenue and have financed our operations with $71.5 million of gross proceeds from sales of our convertible preferred stock and net proceeds of $56.6 million from our initial public offering, or IPO, in October 2015. We do not expect to generate significant revenue unless and until we obtain marketing approval for and commercialize A-101 for the treatment of SK or one of our other future drug candidates.

Since our inception, we have incurred significant operating losses. Our net loss was $8.5 million for the year ended December 31, 2014 and $15.8 million for the nine months ended September 30, 2015. As of September 30, 2015, we had an accumulated deficit of $38.1 million. We expect to incur significant expenses and operating losses for the foreseeable future as we advance our drug candidates from discovery through preclinical development and clinical trials, and seek regulatory approval and pursue commercialization of any approved drug candidate. In addition, if we obtain marketing approval for any of our drug candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. In addition, we may incur expenses in connection with the in-license or acquisition of additional drug candidates. Furthermore, as a result of the IPO, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our drug candidates or delay our pursuit of potential in-licenses or acquisitions.

License Agreement with Rigel

In August 2015, we entered into an exclusive, worldwide license and collaboration agreement with Rigel Pharmaceuticals, Inc., or Rigel, for the development and commercialization of products containing specified JAK inhibitors developed by Rigel. Under this agreement, we intend to develop these JAK inhibitors for the treatment of AA and potentially for other dermatological conditions. Under this agreement, we paid Rigel an upfront non-refundable payment of $8.0 million. In addition, we have agreed to make aggregate payments of up to $80.0 million upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals. Further, we have agreed to pay up to an additional $10.0 million to Rigel upon the achievement of a second set of development milestones. With respect to any products we commercialize under the agreement, we will pay Rigel quarterly tiered royalties on our annual net sales of each product at a high single-digit percentage of annual net sales, subject to specified reductions. The agreement also establishes a joint steering committee composed of an equal number of representatives for each party which will monitor progress in the development of products.

We accounted for the transaction as an asset acquisition as the licensing arrangement did not meet the definition of a business pursuant to the guidance prescribed in Accounting Standards Codification Topic 805, *Business Combinations*. Accordingly, we recorded the $8.0 million upfront payment as research and development expense in the three months ended September 30, 2015. We will record as expense any contingent milestone payments or royalties in the period in which such liabilities are incurred.

Table of Contents

We concluded that the licensing arrangement with Rigel did not meet the definition of a business because the transaction principally resulted in the acquisition of intellectual property. As part of the transaction, we did not acquire any employees or tangible assets, or any processes, protocols or operating systems. In addition, at the time of the acquisition, there were no activities being conducted related to the licensed patents. We will expense the acquired intellectual property assets as of the acquisition date because we will use it in our research and development activities and believe it has no alternative future uses.

Third-Party Agreements

Under an assignment agreement, pursuant to which we acquired intellectual property, we have agreed to pay royalties on sales of A-101 or related products at rates ranging in low single-digit percentages of net sales, as defined in the agreement. Under this assignment agreement, we have paid aggregate milestone payments of $0.2 million and there are no remaining milestone payment obligations under this agreement.

In connection with this acquisition of intellectual property, we also entered into a finder s services agreement under which we have paid aggregate milestone payments of $0.2 million and have agreed to make aggregate payments of up to $1.3 million upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals, as described in the agreement. We have also agreed to make aggregate payments of up to $4.5 million upon the achievement of specified commercial milestones. In addition, we have agreed to pay royalties on sales of A-101 or related products at a low single-digit percentage of net sales, as defined in the agreement.

Components of Our Results of Operations

Revenue

We have not generated any revenue since our inception and do not expect to generate any revenue from the sale of products in the near future.

Research and Development Expenses

Research and development expense consists of expenses incurred in connection with the discovery and development of our drug candidates. We expense research and development costs as incurred. These expenses include:

- expenses incurred under agreements with contract research organizations, or CROs, as well as investigative sites and consultants that conduct our clinical trials and preclinical studies;

- manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical and clinical trial materials and commercial materials, including manufacturing validation batches and related analytical services;

- outsourced professional scientific development services;

- employee-related expenses, which include salaries, benefits and stock-based compensation;

- payments made under a third-party assignment agreement, under which we acquired intellectual property;

- expenses relating to regulatory activities, including filing fees paid to regulatory agencies;

- laboratory materials and supplies used to support our research activities; and

- allocated expenses for utilities and other facility-related costs.

Research and development activities are central to our business model. Drug 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 and long-term toxicology studies. We expect our research and development expenses to increase significantly over the next several years as we increase personnel costs, including stock-based compensation, commence Phase 3 clinical trials of A-101 in patients with SK and conduct other clinical trials and prepare regulatory filings for A-101 and our other drug candidates.

Table of Contents

The successful development of our drug candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of, or when, if ever, material net cash inflows may commence from any of our other drug candidates. This uncertainty is due to the numerous risks and uncertainties associated with the duration and cost of clinical trials, which vary significantly over the life of a project as a result of many factors, including:

- the number of clinical sites included in the trials;
- the length of time required to enroll suitable patients;
- the number of patients that ultimately participate in the trials;
- the duration of the trials;
- the number of doses patients receive;
- the duration of patient follow-up; and
- the results of our clinical trials.

Our expenditures are subject to additional uncertainties, including the terms and timing of regulatory approvals, and the expense of filing, prosecuting, defending and enforcing any patent claims or other intellectual property rights. We may never succeed in achieving regulatory approval for any of our drug candidates. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some drug candidates or focus on others. A change in the outcome of any of these variables with respect to the development of a drug candidate could mean a significant change in the costs and timing associated with the development of that drug candidate. For example, if the FDA or other regulatory authorities were to require us to conduct clinical trials beyond those that we currently anticipate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. Drug commercialization will take several years and millions of dollars in development costs.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for personnel in executive, administrative, finance, legal and business development functions, including stock-based compensation, travel expenses and recruiting expenses. Other general and administrative expenses include facility related costs, patent filing and prosecution costs and professional fees for marketing, legal, auditing and tax services, insurance costs, as well as payments made under our related-party services agreement and milestone payments under our finder s services agreement.

We anticipate that our general and administrative expenses will increase as a result of increased personnel costs, including stock-based compensation, expanded infrastructure and higher consulting, legal and tax-related services associated with maintaining compliance with stock exchange listing and SEC requirements, accounting and investor relations costs, director compensation, and director and officer insurance premiums associated with being a public company. Additionally, if and when we believe a regulatory approval of a drug candidate appears likely, we anticipate an increase in payroll and expense as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our drug candidate.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents and marketable securities.

Critical Accounting Policies and Significant Judgments and Estimates

This management s discussion and analysis of our financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and

Table of Contents

stock-based compensation. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe there have been no material changes to our critical accounting policies and use of estimates as disclosed in the footnotes to our audited financial statements for the year ended December 31, 2014 included in our final prospectus filed on October 8, 2015 with the SEC pursuant to Rule 424(b)(4) of the Securities Act.

Results of Operations

Comparison of Three Months Ended September 30, 2015 and 2014

The following table summarizes our results of operations for the three months ended September 30, 2015 and 2014:

	Three Months Ended September 30,		
	2015	2014	Change
	(In thousands)		
Revenue	$	$	$
Operating expenses:			
Research and development	9,408	2,097	7,311
General and administrative	1,232	565	667
Total operating expenses	10,640	2,662	7,978
Loss from operations	(10,640)	(2,662)	(7,978)
Interest income	7	5	2
Net loss	$ (10,633)	$ (2,657)	$ (7,976)

Research and Development Expenses

Research and development expenses were $9.4 million for the three months ended September 30, 2015, compared to $2.1 million for the three months ended September 30, 2014. The increase of $7.3 million was primarily attributable to an $8.0 million upfront payment to Rigel in connection with our license of rights to JAK inhibitors and related intellectual property, partially offset by a decrease of $0.7 million in direct costs associated with the three Phase 2 clinical trials of our lead drug candidate, A-101 for the treatment of SK, being conducted during the period. This overall decrease in direct costs for the trials consisted of decreases of $0.7 million in clinical expenses and $0.3 million in development-related expenses, partially offset by a $0.2 million increase in manufacturing scale-up expenses.

General and Administrative Expenses

General and administrative expenses were $1.2 million for the three months ended September 30, 2015, compared to $0.6 million for the three months ended September 30, 2014. The increase of $0.6 million was primarily attributable to increases of $0.2 million in payroll-related

expenses due to increased headcount, $0.2 million in market research expenses, $0.1 million in fees related to the formation of our wholly-owned subsidiary, and $0.1 million in professional fees for accounting and auditing services.

Comparison of Nine Months Ended September 30, 2015 and 2014

The following table summarizes our results of operations for the nine months ended September 30, 2015 and 2014:

	Nine Months Ended September 30,		
	2015	2014	Change
	(In thousands)		
Revenue	$	$	$
Operating expenses:			
Research and development	12,937	4,453	8,484
General and administrative	2,928	1,478	1,450
Total operating expenses	15,865	5,931	9,934
Loss from operations	(15,865)	(5,931)	(9,934)
Interest income	16	11	5
Net loss	$ (15,849)	$ (5,920)	$ (9,929)

Table of Contents

Research and Development Expenses

Research and development expenses were $12.9 million for the nine months ended September 30, 2015, compared to $4.5 million for the nine months ended September 30, 2014. The increase of $8.4 million was primarily attributable to the $8.0 million upfront payment to Rigel, as well as an increase of $0.5 million in regulatory expenses, partially offset by a decrease of $0.1 million in direct costs associated with the three Phase 2 clinical trials of A-101 for the treatment of SK. The overall decrease in direct costs for the trials consisted of decreases of $0.7 million in clinical expenses and $0.6 million in development-related expenses, partially offset by an increase of $1.1 million in manufacturing scale-up expenses.

General and Administrative Expenses

General and administrative expenses were $2.9 million for the nine months ended September 30, 2015, compared to $1.5 million for the nine months ended September 30, 2014. The increase of $1.4 million was primarily attributable to increases of $0.5 million in payroll-related expenses due to increased headcount, $0.4 million in market research expenses, $0.2 million in stock-based compensation, $0.1 million in fees related to the formation of our wholly-owned subsidiary, and $0.2 million in professional fees for accounting and auditing services.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue and have incurred net losses and negative cash flows from our operations. We have financed our operations since inception through sales of our convertible preferred stock, receiving aggregate gross proceeds of $71.5 million through September 30, 2015.

As of September 30, 2015, we had cash, cash equivalents and marketable securities of $38.4 million. Subsequent to September 30, 2015, as described below we received cash proceeds of $58.8 million, net of underwriting discounts and commissions, from our IPO. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

We currently have no ongoing material financing commitments, such as lines of credit or guarantees, that are expected to affect our liquidity over the next five years, other than our lease obligations and contingent obligations under intellectual property licensing agreements, each of which are described below.

Initial Public Offering

On October 13, 2015, we closed our IPO in which we sold 5,750,000 shares of common stock at a price to the public of $11.00 per share, for aggregate gross proceeds of $63.3 million. We paid underwriting discounts and commissions of $4.4 million, and we also incurred expenses of

$2.2 million in connection with the offering. As a result, the net offering proceeds to us, after deducting underwriting discounts and commissions and offering expenses, were $56.6 million. See Notes 1, 6 and 14 to our unaudited condensed consolidated financial statements in Part I, Item 1 of this Quarterly Report on Form 10-Q for additional details.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

	Nine Months Ended September 30,	
	2015	**2014**
Cash used in operating activities	$ (16,232)	$ (4,874)
Cash (used in) provided by investing activities	(7,463)	3,586
Cash provided by financing activities	38,357	10,588
Net increase in cash and cash equivalents	$ 14,662	$ 9,300

Table of Contents

Operating Activities

During the nine months ended September 30, 2015, operating activities used $16.2 million of cash, primarily resulting from our net loss of $15.8 million and from cash used by our changes in our operating assets and liabilities of $0.7 million offset by non-cash expenses of $0.3 million. Net cash used in changes in our operating assets and liabilities during the nine months ended September 30, 2015 consisted primarily of a $0.5 million decrease in accounts payable and a $0.8 million increase in prepaid expenses and other current assets, both of which were partially offset by a $0.6 million increase in accrued expenses. The decrease in accounts payable was due to the timing of vendor invoicing and payments. The increase in prepaid expenses and other current assets was primarily due to prepayments for clinical trials. The increase in accrued expenses was due to increases in accruals for payroll and payroll-related costs due primarily to bonuses, as well as increases in accruals for IPO-related expenses incurred.

During the nine months ended September 30, 2014, operating activities used $4.9 million of cash, primarily resulting from our net loss of $5.9 million, partially offset by cash provided by changes in our operating assets and liabilities of $1.0 million. Net cash provided by changes in our operating assets and liabilities during the nine months ended September 30, 2014 consisted primarily of a $0.9 million increase in accounts payable and a $0.5 million increase in accrued expenses, both of which were partially offset by a $0.4 million increase in prepaid expenses and other current assets. The increases in accounts payable and accrued expenses were primarily due to costs related to our clinical trials of A-101. The increase in prepaid expenses and other current assets was primarily due to a prepayment for manufacturing scale-up expenses and clinical trials.

Investing Activities

During the nine months ended September 30, 2015, we used cash of $7.5 million in investing activities, consisting of purchases of marketable securities of $13.0 million and purchases of equipment of $0.4 million, partially offset by proceeds from sales and maturities of marketable securities of $5.9 million.

During the nine months ended September 30, 2014, investing activities provided $3.6 million of cash, consisting of proceeds from sales and maturities of marketable securities of $3.7 million, partially offset by purchases of equipment of $0.1 million.

Financing activities

During the nine months ended September 30, 2015, financing activities provided $38.4 million of cash as a result of $39.9 million in proceeds from the issuance of Series C convertible preferred stock, partially offset by payments of IPO costs of $1.5 million.

During the nine months ended September 30, 2014, financing activities provided $10.6 million of cash from the issuance of Series B convertible preferred stock.

Funding Requirements

We plan to focus in the near term on the development, regulatory approval and potential commercialization of A-101 for the treatment of SK. We anticipate we will incur net losses for the next several years as we complete clinical development of A-101 for the treatment of SK and continue research and development of A-101 for the treatment of common warts, A-102 for the treatment of SK and common warts and A-201 and A-301 for the treatment of AA. In addition, we plan to identify, acquire or in-license and develop additional drug candidates, potentially build commercial capabilities and expand our corporate infrastructure. We may not be able to complete the development and initiate commercialization of these programs if, among other things, our clinical trials are not successful or if the FDA does not approve our drug candidate arising out of our current clinical trials when we expect, or at all.

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, clinical costs, external research and development services, laboratory and related supplies, legal and other regulatory expenses, and administrative and overhead costs. Our future funding requirements will be heavily determined by the resources needed to support development of our drug candidates.

Table of Contents

As a publicly traded company, we will incur significant legal, accounting and other expenses that we were not required to incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, as well as rules adopted by the SEC and The NASDAQ Stock Market, requires public companies to implement specified corporate governance practices that are currently inapplicable to us as a private company. We expect these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

We believe that the net proceeds from our offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months, including the completion of our three planned Phase 3 clinical trials for A-101 for the treatment of SK, the submission of our NDA with the FDA for the approval of A-101 for the treatment of SK in the United States and the completion of our planned Phase 2 clinical trials for A-101 for the treatment of common warts. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. We expect that we will require additional capital to commercialize A-101 for the treatment of SK, if we receive regulatory approval, and to pursue in-licenses or acquisitions of other drug candidates. If we receive regulatory approval for A-101 for the treatment of SK, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution, depending on where we choose to commercialize. Additional funds may not be available on a timely basis, on favorable terms, or at all, and such funds, if raised, may not be sufficient to enable us to continue to implement our long-term business strategy. If we are unable to raise sufficient additional capital, we may need to substantially curtail our planned operations and the pursuit of our growth strategy.

We may raise additional capital through the sale of equity or convertible debt securities. In such an event, the terms of these securities may include liquidation or other preferences that adversely affect the rights of a holder of our common stock.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical drugs, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on many factors, including:

- the number and characteristics of the drug candidates we pursue;

- the scope, progress, results and costs of researching and developing our drug candidates, and conducting preclinical studies and clinical trials;

- the timing of, and the costs involved in, obtaining regulatory approvals for our drug candidates;

- the cost of manufacturing our drug candidates and any drugs we successfully commercialize;

- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;

- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and

- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future drug candidates, if any.

Contractual Obligations and Commitments

We lease office space in Malvern, Pennsylvania under an operating lease agreement that, as amended, requires future rental payments of $0.1 million during the year ending December 31, 2015, an aggregate of $0.4 million during the years ending December 31, 2016 and 2017, and an aggregate of $0.4 million during the years ending December 31, 2018 and 2019.

Under the assignment agreement pursuant to which we acquired intellectual property, we have agreed to pay royalties on sales of A-101 or related products at rates ranging in low single-digit percentages of net sales, as defined in the agreement. Under the related finder s services agreement, we have agreed to make aggregate payments of up to $1.3 million upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals, as described in the agreement. We have also agreed to make aggregate payments of up to $4.5 million upon the achievement of specified commercial milestones. In addition, we have agreed to pay royalties on sales of A-101 or related products at a low single-digit percentage of net sales, as defined in the agreement.

Table of Contents

Under a commercial supply agreement with a third party, we have agreed to pay a termination fee of up to $0.4 million in the event we terminate the agreement without cause or the third party terminates the agreement for cause.

Under a license agreement with Rigel that we entered into in August 2015, we have agreed to make aggregate payments of up to $80.0 million upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals. Further, we have agreed to pay up to an additional $10.0 million to Rigel upon the achievement of a second set of development milestones. With respect to any products we commercialize under the agreement, we will pay Rigel quarterly tiered royalties on our annual net sales of each product developed using the licensed JAK inhibitors at a high single-digit percentage of annual net sales, subject to specified reductions.

We enter into contracts in the normal course of business with CROs for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts generally provide for termination upon notice, and therefore we believe that our non-cancelable obligations under these agreements are not material.

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 in the rules and regulations of the SEC.

Emerging Growth Company Status

The Jumpstart Our Business Startups Act of 2012, or the JOBS Act, permits an emerging growth company such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to opt out of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

Table of Contents

Item 3. Quantitative and Qualitative Disclosures about Market Risk

As of September 30, 2015 and December 31, 2014, we had $25.0 million and $10.0 million of cash equivalents, respectively, composed of overnight money market funds, and we had no debt. As a result, a change in market interest rates would not have any impact on our financial position or results of operations.

Item 4. Controls and Procedures

(a) *Evaluation of Disclosure Controls and Procedures*

The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Security and Exchange Commission s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information is accumulated and communicated to a company s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2015, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of such date at the reasonable assurance level.

(b) *Changes in Internal Controls Over Financial Reporting*

There have not been any changes in our internal controls over financial reporting during our fiscal quarter ended September 30, 2015 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

27

Table of Contents

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we are subject to litigation and claims arising in the ordinary course of business. We are not currently a party to any material legal proceedings and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results, cash flows or financial condition.

Item 1A. Risk Factors

Our business is subject to numerous risks. You should carefully consider the following risks, as well as general economic and business risks, and all of the other information contained in this Quarterly Report on Form 10-Q, together with any other documents we file with the SEC. Any of the following risks could have a material adverse effect on our business, operating results and financial condition and cause the trading price of our common stock to decline.

Risks Related to Our Financial Position and Capital Needs

We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.

We are a clinical-stage specialty pharmaceutical company with limited operating history. Since inception, we have incurred significant net losses. We incurred net losses of $8.5 million and $15.8 million for the year ended December 31, 2014 and the nine months ended September 30, 2015, respectively. As of September 30, 2015, we had an accumulated deficit of $38.1 million. To date, we have financed our operations with $71.5 million in gross proceeds raised in private placements of convertible preferred stock and $56.6 million in aggregate net proceeds from our initial public offering in October 2015. We have no products approved for commercialization and have never generated any revenue.

We have devoted substantially all of our financial resources and efforts to development of our lead drug candidate, A-101 for the treatment of SK, including preclinical studies and clinical trials. We have completed three Phase 2 clinical trials of A-101 in patients with SK. In addition to developing A-101 for the treatment of SK, we are also developing A-101 as a prescription treatment for common warts as well as A-102, a gel dosage form of hydrogen peroxide, as a prescription treatment for SK and common warts. We plan to develop A-201 as an oral treatment for severe forms of AA (alopecia totalis and alopecia universalis) and A-301 as a topical treatment for patchy AA. Therefore, we expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue our ongoing clinical trials evaluating A-101 for the treatment of SK;
- pursue regulatory approvals for A-101 for the treatment of SK and for any other drug candidates that successfully complete clinical trials;
- initiate clinical trials of our other drug candidates, including A-101 for the treatment of common warts, A-102 for the treatment of SK and common warts, and A-201 and A-301 for the treatment of AA;
- seek to discover and develop additional drug candidates;
- ultimately establish a commercialization infrastructure and scale up external manufacturing and distribution capabilities to commercialize any drug candidates for which we may obtain regulatory approval;
- seek to in-license or acquire additional drug candidates for other dermatological conditions;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed drugs;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our drug development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must succeed in developing and eventually commercializing drug candidates that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our drug candidates, obtaining regulatory approval, and manufacturing, marketing and selling any drug candidates for which we may obtain regulatory approval, as well as discovering and developing additional

Table of Contents

drug candidates. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenue that is significant enough to achieve profitability.

In cases where we are successful in obtaining regulatory approval to market one or more of our drug candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement, and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such drug products, even if approved.

Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those expected, or if there are any delays in the initiation and completion of our clinical trials or the development of any of our drug candidates, our expenses could increase.

Even if we 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 depress the value of our company and could impair our ability to raise capital, expand our business, maintain our development efforts, obtain drug approvals, diversify our offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Identifying potential drug candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. We expect to continue to incur significant expenses and operating losses over the next several years as we commence our Phase 3 clinical trials of A-101 in patients with SK, seek marketing approval for A-101 for the treatment of SK and advance our other drug candidates. In addition, our drug candidates, if approved, may not achieve commercial success. Our revenue, if any, will be derived from sales of drugs that we do not expect to be commercially available for a number of years, if at all. If we obtain marketing approval for A-101 for the treatment of SK or any other drug candidates that we develop, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. We also expect an increase in our expenses associated with creating additional infrastructure to support operations as a public company.

As of September 30, 2015, we had cash, cash equivalents and marketable securities of $38.4 million. Subsequent to September 30, 2015, we received net proceeds of $56.6 million from the initial public offering of our common stock. We believe that the net proceeds from our initial public offering, together with our existing cash and cash equivalents as of the date of this report, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities, acquisitions of additional drug candidates, and changes in regulation. Our future capital requirements will depend on many factors, including:

- the progress and results of the three Phase 3 clinical trials of A-101 in patients with SK that we plan to commence in the first quarter of 2016;

- the progress and results of the toxicology studies and Phase 2 clinical trials evaluating A-101 as a potential treatment for common warts;

- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other drug candidates, including A-102, A-201 and A-301;

- the extent to which we in-license or acquire other drug candidates and technologies;

- the number and development requirements of other drug candidates that we may pursue;

- the costs, timing and outcome of regulatory review of our drug candidates;

- the costs and timing of future commercialization activities, including drug manufacturing, marketing, sales and distribution, for any of our drug candidates for which we receive marketing approval;

- the revenue, if any, received from commercial sales of our drug candidates for which we receive marketing approval;

- our ability to establish collaborations to commercialize A-101 outside the United States; and

Table of Contents

- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims.

We expect that we will require additional capital to commercialize A-101 for the treatment of SK. If we receive regulatory approval for A-101 for this indication, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution, depending on where we choose to commercialize. Additional funds may not be available on a timely basis, on favorable terms, or at all, and such funds, if raised, may not be sufficient to enable us to continue to implement our long-term business strategy. If we are unable to raise sufficient additional capital, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or drug candidates.

Until such time, if ever, as we can generate substantial revenue, we may finance our cash needs through a combination of equity offerings, debt financings and license and collaboration agreements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or drug candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

We have a limited operating history and no history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2012, and our operations to date have been largely focused on raising capital and developing A-101 for the treatment of SK, including undertaking preclinical studies and conducting clinical trials. A-101 for the treatment of SK is our only drug candidate for which we have conducted clinical trials. We have not yet demonstrated our ability to successfully complete later-stage clinical trials, obtain regulatory approvals, manufacture a drug on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing drugs.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will need to transition at some point from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Risks Related to the Development of Our Drug Candidates

We are early in our development efforts and have only one drug candidate, A-101 for the treatment of SK, for which we have conducted clinical trials. If we are unable to successfully develop, receive regulatory approval for and commercialize A-101 for the treatment of SK or any other drug candidates, or experience significant delays in doing so, our business will be harmed.

We currently have no drug products that are approved for commercial sale. We are early in our development efforts and have only one drug candidate, A-101 for the treatment of SK, for which we have conducted Phase 2 clinical trials. We have not completed the development of any drug candidates and we may never be able to develop marketable drugs. We have invested substantially all of our efforts and financial resources in the development of A-101 for the treatment of SK, the development of our other drug candidates and the identification of potential drug candidates. Our ability to generate revenue from our drug candidates, which we do not expect will occur for a number of years, if ever, will depend heavily on their successful development, regulatory approval and eventual commercialization of these drug candidates. The success of A-101 or any other drug candidates that we develop, including A-102, A-201 and A-301, will depend on several factors, including:

Table of Contents

- successful completion of preclinical studies and our clinical trials;
- successful development of our manufacturing processes for any of our drug candidates that receive regulatory approval;
- receipt of timely marketing approvals from applicable regulatory authorities;
- launching commercial sales of drugs, if approved;
- acceptance of our drugs, if approved, by patients, the medical community and third-party payors, and willingness of patients to pay out of pocket for procedures using our drug candidates for the treatment of SK;
- our success in educating physicians and patients about the benefits, administration and use of A-101 or any other drug candidates, if approved;
- the prevalence and severity of adverse events experienced with A-101 or our other drug candidates;
- the availability, perceived advantages, cost, safety and efficacy of alternative treatments for SK;
- obtaining and maintaining patent, trademark and trade secret protection and regulatory exclusivity for our drug candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintaining compliance with regulatory requirements, including current good manufacturing practices, or cGMPs;
- competing effectively with other procedures; and
- maintaining a continued acceptable safety, tolerability and efficacy profile of the drugs following approval.

Whether regulatory approval will be granted is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities. Our drug candidates success in clinical trials will not guarantee regulatory approval. If, following submission, our NDA for A-101 for the treatment of SK or any other drug candidate is not accepted for substantive review, or even if it is accepted for substantive review, the FDA or other comparable foreign regulatory authorities may require that we conduct additional studies or clinical trials, provide additional data, take additional manufacturing steps, or require other conditions before they will reconsider or approve our application. If the FDA or other comparable foreign regulatory authorities require additional studies, clinical trials or data, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, the FDA or other comparable foreign regulatory authorities may not consider sufficient any additional required studies, clinical trials, data or information that we perform and complete or generate, or we may decide to abandon the program.

It is possible that A-101 or any of our other drug candidates will never obtain regulatory approval, even if we expend substantial time and resources seeking such approval. 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 drug candidates, which would harm our business.

Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.

The risk of failure for our drug candidates is high. It is impossible to predict when or if any of our drug candidates will prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our drug candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs.

We have not completed all clinical trials required for the approval of any of our drug candidates. Based on the feedback from our meeting with the FDA in May 2015, we plan to commence three Phase 3 clinical trials of A-101 in patients with SK lesions on the face, trunk and extremities in the first quarter of 2016. We have also received written guidance from the EMA regarding the design of our Phase 3 clinical trials for A-101 for the treatment of SK. The development of our other drug candidates is less advanced and we have not commenced any clinical trials. We cannot assure you that any Phase 3 or other clinical trials that we may conduct will demonstrate consistent or adequate efficacy and safety to obtain regulatory approval to market our drug candidates.

Table of Contents

We may experience numerous unforeseen events during or as a result of clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our drug candidates, including:

- 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 experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- clinical trials of our drug candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;

- the number of patients required for clinical trials of our drug 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 or fail to return for post-treatment follow-up at a higher rate than we anticipate;

- our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials;

- 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 require that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;

- the cost of clinical trials of our drug candidates may be greater than we anticipate; and

- the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the institutional review boards of the institutions in which such trials are being conducted, by the data safety monitoring board for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, if the FDA does not support our proposed design and clinical endpoints for our Phase 3 clinical trials for A-101, our clinical trials could be delayed. If we experience delays in the completion of, or termination of, any clinical trial of our drug candidates, the commercial prospects of our drug candidates will be harmed, and our ability to generate product revenues from any of these drug candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our drug candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may

also ultimately lead to the denial of regulatory approval of our drug candidates. If we are required to conduct additional clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our drug candidates or other testing, if the results of these trials or tests are not favorable or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Our drug development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring drugs to market before we do and impair our ability to successfully commercialize our drug candidates.

Table of Contents

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population. Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including:

- the eligibility criteria for the trial in question;
- the perceived risks and benefits of the drug candidate in the trial;
- the availability of drugs approved to treat the skin disease in the trial;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us or them to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our drug candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on and expect to continue to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

Our clinical trials may fail to demonstrate the safety and efficacy of our drug candidates, or serious adverse or unacceptable side effects may be identified during the development of our drug candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our drug candidates.

Before obtaining regulatory approvals for the commercial sale of our drug candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our drug candidates are both safe and effective for use in each target indication, and failures can occur at any stage of testing. Clinical trials often fail to demonstrate safety and efficacy of the drug candidate studied for the target indication.

If our drug candidates are associated with side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. The FDA or an institutional review board may also require that we suspend, discontinue, or limit our clinical trials based on safety information. Such findings could further result in regulatory authorities failing to provide marketing authorization for our drug candidates. Many drug candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented further development of the drug candidate.

Additionally, if one or more of our drug candidates receives marketing approval, and we or others identify undesirable side effects caused by such drugs, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the labels;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients; and
- our reputation and physician or patient acceptance of our products may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular drug candidate, if approved, and could significantly harm our business, results of operations and prospects.

Table of Contents

Changes in methods of drug candidate manufacturing or formulation may result in additional costs or delay.

As drug candidates are developed through preclinical studies to late-stage clinical trials towards approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our drug candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. For example, if we need to manufacture A-102, we may experience difficulties manufacturing a stable gel dosage form as opposed to a topical solution. Such changes may also require additional testing, FDA notification or FDA approval. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our drug candidates and jeopardize our ability to commence sales and generate revenue.

We may not be successful in our efforts to increase our pipeline of drug candidates, including by in-licensing or acquiring additional drug candidates for other dermatological conditions.

A key element of our strategy is to build and expand our pipeline of drug candidates. In addition, we intend to in-license or acquire additional drug candidates for other dermatological conditions to build a fully integrated dermatology company. We may not be able to identify or develop drug candidates that are safe, tolerable and effective. Even if we are successful in continuing to build our pipeline, the potential drug candidates that we identify, in-license or acquire may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance.

We may expend our limited resources to pursue a particular drug candidate or indication and fail to capitalize on drug candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we focus on development programs and drug candidates that we identify for specific indications. As such, we are currently primarily focused on the development of A-101 for the treatment of SK. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future development programs and drug candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

Risks Related to the Commercialization of Our Drug Candidates

Even if any of our drug candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our drug candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our drug candidates do not achieve an adequate level of acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our drug candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy, safety and potential advantages compared to alternative treatments;
- our ability to offer our drugs for sale at competitive prices;
- the ability of dermatologists to charge a premium for A-101 and our other drug candidates;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new treatments and of physicians to prescribe these treatments;
- our ability to hire and retain a sales force in the United States;
- the strength of marketing and distribution support;
- the willingness of patients to pay out of pocket for procedures using A-101 for the treatment of SK;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our drugs together with other medications.

Table of Contents

If we are unable to establish sales, marketing and distribution capabilities for A-101 or any other drug candidate that may receive regulatory approval, we may not be successful in commercializing those drug candidates if and when they are approved.

We do not have sales or marketing infrastructure. To achieve commercial success for A-101 and any other drug candidate for which we may obtain marketing approval, we will need to establish a sales and marketing organization. In the future, we expect to build a focused sales and marketing infrastructure to market or co-promote some of our drug candidates in the United States, if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time consuming and could delay any drug launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drugs on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and enter into arrangements with third parties to perform these services, our revenue and our profitability, if any, are likely to be lower than if we were to sell, market and distribute any drugs that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our drug candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drugs effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug candidates.

We face substantial competition, which may result in others discovering, developing or commercializing drugs before or more successfully than we do.

The development and commercialization of new drugs is highly competitive. We face competition with respect to our current drug candidates, and will face competition with respect to any drug candidates that we may seek to develop or commercialize in the future, from many different sources, including major pharmaceutical and specialty pharmaceutical companies, academic institutions and governmental agencies and public and private research institutions.

With respect to A-101 for the treatment of SK, we are aware of one biopharmaceutical company developing a combination drug candidate that targets SK, and another company that currently markets a line of cosmetic products targeting skin conditions, including SK.

With respect to A-101 for the treatment of common warts, we are aware of one company developing a prescription treatment for common warts and another company that intends to initiate a Phase 2 clinical trial of a gel as a prescription treatment for common warts. In addition, other drugs have been used off-label as treatments for common warts. We could also encounter competition from over-the-counter treatments for common warts.

With respect to A-201 and A-301 for the treatment of AA, we anticipate competing with sensitizing agents such as diphencyprone, or DPCP, and topical, intralesional and systemic corticosteroids, which have been found to occasionally reduce symptoms of AA. Other treatments utilized for patchy AA include anthralin and minoxidil solution. We may also compete with companies developing chemical agents to be used in topical immunotherapies, as well as companies developing biologics, immunosuppressive agents, laser therapy, phototherapy, other JAK inhibitors and prostaglandin analogues to treat AA.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than A-101 or any

Table of Contents

other drug that we may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs more rapidly than we may obtain approval for our drug, which could result in our competitors establishing a strong market position before we are able to enter the market.

Many of the companies against which we are competing, or against which we may compete in the future, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or that may be necessary for, our programs.

We expect third-party payors generally will not cover the use of our drug candidates for the treatment of SK and, accordingly, our success will be dependent upon the willingness of patients to pay out of pocket for procedures using these drug candidates.

We do not expect third-party payors to cover and reimburse providers who use A-101 or A-102 on patients for the treatment of SK. Payors generally do not reimburse the provider for the product used to remove non-malignant lesions, including SK. In addition, they do not generally reimburse providers for the procedure removing such lesions, since the procedure is considered to be cosmetic in nature, unless there is a medical need to remove the lesion such as confirming a diagnosis with a biopsy or treating SK that are causing the patient physical discomfort. We anticipate that in some cases, our drug candidates will be used to remove SK lesions that are inflamed and causing the patient discomfort. Any reduction in reimbursement for the procedure to remove inflamed SK may result in a higher percentage of patients needing to pay out of pocket for treatment with our drug candidates. Accordingly, the commercial success of A-101 and A-102 depends on the extent to which patients will be willing to pay out of pocket for the in-office procedure using these drug candidates.

The success of our drug candidates for the treatment of common warts will depend significantly on continued coverage and adequate reimbursement or the willingness of patients to pay for these procedures.

In the case of A-101 and A-102 for the treatment of common warts, we believe our success depends on continued coverage and adequate reimbursement for in-office wart treatment procedures or, in the absence of coverage and adequate reimbursement, on the extent to which patients will be willing to pay out of pocket for the in-office procedures that include our drug candidates.

Third-party payors determine which medical procedures they will cover and establish reimbursement levels. Even if a third-party payor covers a particular procedure, the resulting reimbursement payment rates may not be adequate. Patients who are treated in-office for a medical condition generally rely on third-party payors to reimburse all or part of the costs associated with the procedure and may be unwilling to undergo such procedures for the removal of warts in the absence of such coverage and reimbursement. Physicians may be unlikely to offer procedures for the treatment of warts if they are not covered by insurance and may be unlikely to purchase and use our product for warts unless coverage is provided and reimbursement is adequate.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor s determination that a procedure is neither cosmetic, experimental, nor investigational; safe, effective, and medically necessary; appropriate for the specific patient; cost-effective;

supported by peer-reviewed medical journals; and included in clinical practice guidelines.

Further, from time to time, typically on an annual basis, payment rates are updated and revised by third-party payors. To the extent that the procedures using our drug candidates, if approved, are covered, the cost of our products are generally recovered by the healthcare provider as part of the payment for performing a procedure and not separately reimbursed. Accordingly, these updates could impact the demand for our drug candidates, if approved. An example of payment updates is the Medicare program updates to physician payments, which is done on an annual basis using a prescribed statutory formula. In the past, when the application of the formula resulted in lower payment, Congress has passed interim legislation to prevent the reductions. Most recently, the Protecting Access to Medicare Act of 2014, signed into law in April 2014, provided for a 0.5% update from 2013 payment rates under the Medicare Physician Fee Schedule through 2014 and a 0% update from January 1 until March 31, 2015. If Congress fails to intervene to prevent the negative update factor in future years, the resulting decrease in payment may adversely affect our revenue and results of operations. In addition, the Medicare physician

Table of Contents

fee schedule has been adapted by some private payors into their plan-specific physician payment schedule. We cannot predict how pending and future healthcare legislation will impact our business, and any changes in coverage and reimbursement that further restricts coverage of our drug candidates or lowers reimbursement for procedures using our products could harm our business.

Foreign governments also have their own healthcare reimbursement systems, which vary significantly by country and region, and we cannot be sure that coverage and adequate reimbursement will be made available with respect to the treatments in which our drugs are used under any foreign reimbursement system.

There can be no assurance that our drug candidates for the treatment of common warts, if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary, that they will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available, or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our drugs candidates profitably if they are approved for sale.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any drugs that we may develop.

We face an inherent risk of product liability exposure related to the testing of our drug candidates in human clinical trials and will face an even greater risk if we commercially sell any drugs that we may develop. If we cannot successfully defend ourselves against claims that our drug candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidates or drugs that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any drugs that we may develop.

We currently hold $5.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $5.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our drug candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Our business and operations would suffer in the event of computer system failures, cyber-attacks or a deficiency in our cyber-security.

Despite the implementation of security measures, our internal computer systems, and those of third parties on which we rely, are vulnerable to damage from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, cyber-attacks or cyber-intrusions over the Internet, attachments to emails, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. 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 was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability, damage to our reputation, and the further development of our drug candidates could be delayed.

Table of Contents

Risks Related to Our Dependence on Third Parties

We will rely on third parties to conduct our future clinical trials for drug candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We have engaged a CRO to conduct our planned clinical trials of A-101 and expect to engage a CRO to conduct clinical trails of our other drug candidates that may progress to clinical development. We expect to continue to rely on third parties, such as clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or 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, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our drug candidates. Consequently, our results of operations and the commercial prospects for our drug candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly.

Switching or adding CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not control their activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, or GCPs, 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. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

We also expect to 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 marketing approval of our drug candidates or commercialization of our drugs, producing additional losses and depriving us of potential revenue.

We contract with third parties for the manufacture of A-101 for preclinical and clinical testing and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of A-101 or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities or personnel. We currently rely, and expect to continue to rely, on third parties for the manufacture of A-101 for preclinical and clinical testing, as well as for commercial manufacture if any of our drug candidates, including A-101, receive marketing approval. For example, we have entered into an exclusive, ten-year, automatically renewable supply agreement with PeroxyChem, a manufacturer of hydrogen peroxide, to provide the active pharmaceutical ingredient that can be used in A-101 for the treatment of SK. This reliance on third parties increases the risk that we will not have sufficient quantities of A-101 or such quantities at an acceptable cost or quality, which could delay, prevent or impair our ability to timely conduct our clinical trials or our other development or commercialization efforts.

We also expect to rely on third-party manufacturers or third-party collaborators for the manufacture of commercial supply of A-101 or any other drug candidates for which we obtain marketing approval. The facilities used by our contract manufacturers to manufacture our drug candidates must be approved by the FDA or other regulatory authorities pursuant to inspections that will be conducted after we submit our NDA or comparable marketing application to the FDA or other regulatory authority. We do not have control over a supplier s or manufacturer s compliance with laws, regulations and applicable cGMP standards and other laws and regulations, such as those related to environmental health and safety matters.

Table of Contents

If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure and maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our drug candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our drug candidates, if approved.

We may be unable to establish any agreements with future third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possible increase in costs by PeroxyChem for the active pharmaceutical ingredient in A-101; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our drugs.

Our drug candidates and any drugs that we may develop may compete with other drug candidates and drugs for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for the components of A-101.

If our current contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers. We may incur added costs and delays in identifying and qualifying any such replacement.

We expect to continue to depend on third-party contract manufacturers for the foreseeable future. Our current and anticipated future dependence upon others for the manufacture of our drug candidates or drugs may adversely affect our future profit margins and our ability to commercialize any drugs that receive marketing approval on a timely and competitive basis.

We may seek collaborations with third parties for the development or commercialization of our drug candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these drug candidates.

We may seek third-party collaborators for the development and commercialization of our drug candidates, including for the commercialization of any of our drug candidates that are approved for marketing outside the United States. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our drug candidates. Our ability to generate revenue from these arrangements will depend on our collaborators abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our drug candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any drug candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial

Table of Contents

results, changes in the collaborators strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;

- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our drug candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- drug candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own drug candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of our drug candidates;

- a collaborator with marketing and distribution rights to one or more of our drug candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such drugs;

- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of drug candidates, might lead to additional responsibilities for us with respect to drug candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;

- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable drug candidates.

Collaboration agreements may not lead to development or commercialization of drug candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our drug development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our drug candidates will require substantial additional capital. For some of our drug candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential

commercialization of those drug candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator s evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject drug candidate, the costs and complexities of manufacturing and delivering such drug candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative drug candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our drug candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such drug candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our drug candidates or bring them to market and generate revenue.

Table of Contents

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology and drug candidates may be impaired.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our drug candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our drug candidates.

The patent prosecution process is expensive and time-consuming, however, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we or our licensors were the first to make the inventions claimed in our patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our technology or drugs, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, we may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or drugs and compete directly with us, without payment to

us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications that we own or license is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

Even if our patent applications that we own or license issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or drugs in a non-infringing manner.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged 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 drugs, or

Table of Contents

limit the duration of the patent protection of our technology and drugs. Our issued U.S. patents, with claims directed to treatment of SK and acrochordons with A-101, are scheduled to expire in 2022. Certain issued U.S. patents relating to our JAK inhibitors, A-201 and A-301, are scheduled to expire in 2023 and additional U.S. patents, with claims specifically directed to our JAK inhibitors, are scheduled to expire in 2030. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our issued patents or other intellectual property. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents or that our patents are invalid or unenforceable. In a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent s claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. We may find it impractical or undesirable to enforce our intellectual property against some third parties. For instance, we are aware of third parties that have marketed high-concentration hydrogen peroxide solutions over the internet for the treatment of SK. These parties do not appear to have regulatory authority, and we have not authorized them in any way to market these products. However, to date we have refrained from seeking to enforce our intellectual property rights against these third parties due to the transient nature of their activities. With respect to A-201 and A-301, if we do not elect to exercise our first right to do so, Rigel may enforce the licensed patents relating to A-201 and A-301 against any infringing third party in the field of dermatology. In addition, Rigel has the first right, but not the obligation, to enforce the licensed patents relating to A-201 and A-301 against any infringing party outside of the field of dermatology.

If we breach our license and collaboration agreement with Rigel, it could compromise our development and commercialization efforts for our JAK inhibitors.

In August 2015, we entered into an exclusive license and collaboration agreement with Rigel, which grants us the rights to certain patent rights and other intellectual property owned by Rigel relating to our JAK inhibitors. If we materially breach or fail to perform any provision under this license agreement, including failure to make payments to Rigel when due for royalties and failure to use commercially reasonable efforts to develop and commercialize a JAK inhibitor, Rigel has the right to terminate our license, and upon the effective date of such termination, our right to practice the licensed Rigel patent rights and other intellectual property would end. Any uncured, material breach under the license agreement could result in our loss of rights to practice the patent rights and other intellectual property licensed to us under the license and collaboration agreement, and, to the extent such patent rights and other technology relate to our JAK inhibitors, it could compromise our development and commercialization efforts for A-201 or A-301.

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

Filing, prosecuting and defending patents on our drug candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. For example, the use of A-101 for the treatment of SK is currently covered in patents in the United States, Australia, India and New Zealand, but not in the European

Union or other countries. Our JAK inhibitors being used in the development of A-201 and A-301 are currently covered in patents and applications in the United States, Australia, Brazil, Canada, Chile, China, Eurasia, the European Union, Hong Kong, Israel, India, Japan, South Korea, Mexico, Malaysia, New Zealand, Peru, Singapore, Ukraine, Vietnam, and South Africa. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our invention in such countries. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our drug candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Table of Contents

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. 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, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of our drug candidates. For example, we exclusively license intellectual property from Rigel in the field of dermatology related to our JAK inhibitors. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially.

Rigel or a sublicensee may develop our JAK inhibitors outside of the field of dermatology or another JAK inhibitor.

We exclusively license intellectual property from Rigel in order to develop, use, manufacture, sell and commercialize our JAK inhibitors in the field of dermatology. Rigel retained the rights under such intellectual property to develop, use, manufacture, sell and commercialize such JAK inhibitors outside of the field of dermatology. If Rigel, or a sublicensee, does commercialize such JAK inhibitors outside the field of dermatology, such a product could possibly be used off-label for a dermatology indication, which could negatively impact sales of our JAK inhibitor product candidates, if approved. Rigel also retained the intellectual property rights to develop, use, manufacture, sell and commercialize other structurally similar JAK inhibitors. If Rigel, or a sublicensee, does commercialize a structurally similar JAK inhibitor, such a product could directly compete with our product candidates, if approved.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability to develop, manufacture, market and sell our drug candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our drugs and technology, including interference or derivation proceedings before the USPTO. Numerous U.S. and foreign issued patents and pending patent applications owned by third parties exist in the fields in which we are developing our drug

candidates. For example, we are aware of third parties that are pursuing broad claims directed to the use of JAK inhibitors for the treatment of AA. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party s intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our drugs and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could 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 drug. In addition, 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 drug candidates or force us to cease some of our business operations. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys fees for willful infringement, pay royalties, redesign our infringing product or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Table of Contents

We may be subject to claims by third parties asserting that we, our employees or our licensor have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees and our licensor s employees were previously employed at other biotechnology or pharmaceutical companies. Although we and our licensor try to ensure that our employees and our licensor s employees do not use the proprietary information or know-how of others in their work for us, we or our licensor may be subject to claims that these employees, our licensor or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee s former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees 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. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensor fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we and our licensor are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time-consuming and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court.

If we were to initiate legal proceedings against a third party to enforce a patent directed to our drug candidates, or one of our future drug candidates, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our drug candidates. Such a loss of patent protection would harm our business.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms.

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 this type of litigation.

Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, or in-license needed technology or other drug candidates. There could also be public announcements of the results of the hearing, motions, or other interim proceedings or developments. If securities analysts or investors perceive those results to be negative, it could cause the price of shares of our common stock to decline.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities.

Table of Contents

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 substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. 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 compromise our ability to compete in the marketplace, including compromising our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development collaborations that would help us commercialize our drug candidates, if approved.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our drug candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

The validity, scope and enforceability of any patents listed in the Orange Book that cover A-101 and our JAK inhibitors can be challenged by competitors.

If A-101 or one of our JAK inhibitors is approved by the FDA, one or more third parties may challenge the patents covering A-101 or our JAK inhibitors, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or a finding of non-infringement. For example, if a third party files an Abbreviated New Drug Application, or ANDA, for a generic drug containing A-101, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify to the FDA that either: (1) there is no patent information listed in the FDA s Orange Book with respect to our NDA for the applicable approved drug candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of the third party s generic drug. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved drug candidate, or that such patents are invalid, is called a paragraph IV certification. If the third party submits a paragraph IV certification to the FDA, a notice of the paragraph IV certification must also be sent to us once the third party s ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party s ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party s ANDA will not be subject to the 30-month stay of FDA approval. Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management s attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with our drug candidates.

If we do not obtain protection under the Hatch-Waxman Amendments by extending the patent term and obtaining data exclusivity for our drug candidates, our business may be materially harmed.

Our commercial success will largely depend on our ability to obtain and maintain patent and other intellectual property in the United States and other countries with respect to our proprietary technology, drug candidates and our target indications. Our issued U.S. patents, with claims directed to treatment of SK and acrochordons with A-101, are scheduled to expire in 2022. Certain issued U.S. patents relating to our JAK inhibitors, A-201 and A-301, are scheduled to expire in 2023 and additional U.S. patents, with claims specifically directed to our JAK inhibitors, are scheduled to expire in 2030. Given the

Table of Contents

amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting our drug candidates might expire before or shortly after such candidates begin to be commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents.

Depending upon the timing, duration and specifics of FDA marketing approval of our drug 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 beyond the normal expiration of the patent as compensation for patent term lost during development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). This extension is limited to only one patent that covers the approved product. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request.

If we are unable to extend the expiration date of our existing patents or obtain new patents with longer expiry dates, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing products following our patent expiration and launch their product earlier than might otherwise be the case.

Any trademarks we have obtained or may obtain may be infringed or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish any of our drug candidates that are approved for marketing from the products of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks.

Outside of the United States we cannot be certain that any country s patent or trademark office will not implement new rules that could seriously affect how we draft, file, prosecute and maintain patents, trademarks and patent and trademark applications.

We cannot be certain that the patent or trademark offices of countries outside the United States will not implement new rules that increase costs for drafting, filing, prosecuting and maintaining patents, trademarks and patent and trademark applications or that any such new rules will not restrict our ability to file for patent protection. For example, we may elect not to seek patent protection in some jurisdictions or for some drug candidates in order to save costs. We may be forced to abandon or return the rights to specific patents due to a lack of financial resources.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make formulations or compositions that are the same as or similar to A-101 but that are not covered by the claims of the patents that we own;

- others may be able to make a JAK inhibitor that is similar to the JAK inhibitors we licensed from Rigel that is not covered by the patents that we exclusively licensed and have the right to enforce;

- we, our licensor or any collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own;

- we, our licensor might not have been the first to file patent applications covering certain of our inventions;

- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

Table of Contents

- it is possible that our pending patent applications will not lead to issued patents;

- issued patents that we own may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;

- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets; and

- we may not develop additional proprietary technologies that are patentable.

Risks Related to Regulatory Approval of Our Drug Candidates and Other Legal Compliance Matters

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our drug candidates, and our ability to generate revenue will be materially impaired.

Our drug candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the European Commission and EU Member State Competent Authorities and similar regulatory authorities outside the United States. Failure to obtain marketing approval for a drug candidate will prevent us from commercializing the drug candidate. We have not received approval to market any of our drug candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the drug candidate s safety and efficacy. Securing marketing approval also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our drug candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug in this way, which could limit sales of the drug.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive and may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the drug candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted drug application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a drug candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved drug not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of our drug candidates, the commercial prospects for our drug candidates may be harmed and our ability to generate revenue will be materially impaired.

Failure to obtain marketing approval in international jurisdictions would prevent our drug candidates from being marketed abroad.

In order to market and sell our drugs in the European Union and any other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the drug be approved for reimbursement before the drug can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, failure to obtain approval in one jurisdiction may impact our ability to obtain approval elsewhere. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our drugs in any market.

Table of Contents

A variety of risks associated with marketing our drug candidates internationally could harm our business.

We may seek regulatory approval for A-101 and our other drug candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- 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;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign reimbursement, pricing and insurance regimes;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- 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.

These and other risks associated with our international operations may compromise our ability to achieve or maintain profitability.

Any drug candidate for which we obtain marketing approval could be subject to post-marketing restrictions or recall or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our drug candidates, when and if any of them are approved.

Any drug candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such drug candidate, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a drug candidate is granted, the approval may be subject to limitations on the indicated uses for which the drug candidate may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the drug.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the drug. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers communications regarding off-label use and if we do not market our drugs for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our drugs, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

- restrictions on such drugs, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- restrictions on drug distribution or use;
- requirements to conduct post-marketing studies or clinical trials;

Table of Contents

- warning letters;
- recall or withdrawal of the drugs from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- clinical holds;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our drugs;
- drug seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with the European Union s requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of drugs for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union s requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our current and future relationships with third-party payors, health care professionals and customers in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, physician payment transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to significant penalties.

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our future arrangements with third-party payors, health care professionals and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, that may constrain the business or financial arrangements and relationships through which we sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by the federal government and by the U.S. states and foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation. Moreover, the government may assert that a claim including items or services resulting

from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or *qui tam* actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal Open Payments program, created under Section 6002 of Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the Affordable Care Act, and its implementing regulations, which requires specified manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children s Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other transfers of value made to physicians, which is defined to include doctors, dentists,

Table of Contents

optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers to report annually to CMS ownership and investment interests held by the physicians and their immediate family members by the 90th day of each calendar year. All such reported information is publicly available; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including our relationships with physicians and other healthcare providers, some of whom may recommend, purchase and/or prescribe our drug candidates, if approved, may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. By way of example, some of our consulting arrangements with physicians may not meet all of the criteria of the personal services safe harbor under the federal Anti-Kickback Statute. Accordingly, they may not qualify for safe harbor protection from government prosecution. A business arrangement that does not substantially comply with a safe harbor, however, is not necessarily illegal under the Anti-Kickback Statute, but may be subject to additional scrutiny by the government.

If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our drug candidates and 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 marketing approval of our drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any drug candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, President Obama signed into law the Affordable Care Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance

industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of the Affordable Care Act of importance to our potential drug candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, which include new government investigative powers and enhanced penalties for non-compliance;

Table of Contents

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer s outpatient drugs to be covered under Medicare Part D;

- extension of manufacturers Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals, thereby potentially increasing manufacturers Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- the new requirements under the federal Open Payments program and its implementing regulations;

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. These changes included aggregate reductions to Medicare payments to providers of 2% per fiscal year effective April 1, 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and, accordingly, our financial operations.

We expect that the Affordable Care Act, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs.

Additionally, new litigation challenging the federal tax subsidies received by individuals to purchase health insurance under the Affordable Care Act is currently pending before the U.S. Supreme Court that could affect our business. Final regulations, guidance, and judicial orders are anticipated in the near future and we will continue to assess the Affordable Care Act s impact on us as final regulations, guidance, and orders are issued.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. We cannot be 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 drug 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 drug labeling and post-marketing testing and other requirements.

We may not be able to obtain five-year FDA regulatory exclusivity as an NCE.

The FDA provides periods of regulatory exclusivity following their approval of an NDA, which provide the holder of an approved NDA limited protection from new competition in the marketplace for the innovation represented by its approved drug. Five-year exclusivity precludes approval of 505(b)(2) applications or ANDAs by delaying the submission or approval of such applications, while three-year exclusivity precludes the approval of such applications. We intend to seek new chemical entity, or NCE, status for A-101, and we may seek NCE status for other drug candidates as appropriate. Five years of exclusivity are available to NCEs following the approval of an NDA by the FDA. An NCE is a drug that contains no active moiety that has been approved by FDA in any other NDA. If a drug is not eligible for the NCE exclusivity, it may be eligible for three years of exclusivity. Three-year exclusivity is available to the holder of an NDA for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials, other than bioavailability or bioequivalence trials, were essential to the approval of the application and were conducted or sponsored by the applicant.

There is a risk that the FDA may disagree with any claim that we may make that A-101 or any of our other drug candidates are NCEs and therefore entitled to five-year exclusivity.

Table of Contents

If we do obtain either five or three years of exclusivity, such exclusivity will not block all potential competitors from the market. Five-year exclusivity does not block complete 505(b)(1) NDAs and the scope of three-year exclusivity is limited to the conditions for use approved in the NDA.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available procedures. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

The inherent dangers in production and transportation of hydrogen peroxide could cause disruptions and could expose us to potentially significant losses, costs or liabilities.

Our operations are subject to significant hazards and risks inherent in the use and transport of hydrogen peroxide, the active ingredient of A-101 and A-102. Hydrogen peroxide can decompose in the presence of organic materials and is categorized as an oxidizer and is corrosive. Hydrogen

peroxide should be stored in cool, dry, well-ventilated areas and away from any flammable or combustible substances. The hazards and risks associated with producing and transporting hydrogen peroxide include fires, explosions, third-party interference (including terrorism) and mechanical failure of equipment at our facilities or those of our supplier of hydrogen peroxide. The occurrence of any of these events could result in production and distribution difficulties and disruptions, personal injury or wrongful death claims and other damage to properties.

We are subject to governmental economic sanctions and export and import controls that could impair our ability to compete in international markets or subject us to liability if we are not in compliance with applicable laws.

As a U.S. company, we are subject to U.S. import and export controls and economic sanctions laws and regulations, and we are required to import and export our drug candidates, technology and services in compliance with those laws and regulations, including the U.S. Export Administration Regulations, the International Traffic in Arms Regulations, and economic embargo and trade sanction programs administered by the Treasury Department s Office of Foreign Assets Control.

U.S. economic sanctions and export control laws and regulations prohibit the shipment of certain products and services to countries, governments and persons targeted by U.S. sanctions. While we are currently taking precautions to prevent doing any business, directly or indirectly, with countries, governments and persons targeted by U.S. sanctions and to ensure that our

Table of Contents

drug candidates, if approved, are not exported or used by countries, governments and persons targeted by U.S. sanctions, such measures may be circumvented.

Furthermore, if we export our drug candidates, if approved, the exports may require authorizations, including a license, a license exception or other appropriate government authorization. Complying with export control and sanctions regulations for a particular sale may be time-consuming and may result in the delay or loss of sales opportunities. Failure to comply with export control and sanctions regulations for a particular sale may expose us to government investigations and penalties.

If we are found to be in violation of U.S. sanctions or import or export control laws, it could result in civil and criminal, monetary and non-monetary penalties, including possible incarceration for those individuals responsible for the violations, the loss of export or import privileges and reputational harm.

We are subject to anti-corruption and anti-money laundering laws with respect to our operations and non-compliance with such laws can subject us to criminal and/or civil liability and harm our business.

We are subject to the U.S. Foreign Corrupt Practices Act of 1977, as amended, or the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act and possibly other anti-bribery and anti-money laundering laws in countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees and third-party intermediaries from authorizing, offering or providing, directly or indirectly, improper payments or benefits to recipients in the public or private sector. As we commercialize our drug candidates and eventually commence international sales and business, we may engage with collaborators and third-party intermediaries to sell our products abroad and to obtain necessary permits, licenses and other regulatory approvals. We or our third-party intermediaries may have direct or indirect interactions with officials and employees of government agencies or state-owned or affiliated entities. We can be held liable for the corrupt or other illegal activities of these third-party intermediaries, our employees, representatives, contractors, partners and agents, even if we do not explicitly authorize such activities.

Noncompliance with anti-corruption and anti-money laundering laws could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, other enforcement actions, disgorgement of profits, significant fines, damages, other civil and criminal penalties or injunctions, suspension and/or debarment from contracting with certain persons, the loss of export privileges, reputational harm, adverse media coverage and other collateral consequences. Responding to any action will likely result in a materially significant diversion of management s attention and resources and significant defense costs and other professional fees.

Risks Related to Employee Matters and Managing Our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, development, clinical, financial and business development expertise of Dr. Neal Walker, our Chief Executive Officer, Christopher Powala, our Chief Operating Officer, Dr. Stuart Shanler, our Chief Scientific Officer, Frank Ruffo, our Chief

Financial Officer, and Kamil Ali-Jackson, our Chief Legal Officer, as well as the other members of our scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may currently terminate their employment with us at any time. We do not maintain key person insurance for any of our executives or employees other than Dr. Walker and Mr. Powala.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our drug pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our 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. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Table of Contents

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of September 30, 2015, we had 11 full-time employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and, if any of our drug candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, 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. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter 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 be in compliance with such 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 civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations.

Risks Related to Ownership of Our Common Stock

An active trading market for our common stock may not continue to develop or be sustained.

Prior to our initial public offering in October 2015, there was no public market for our common stock. Although our common stock is listed on The NASDAQ Global Select Market, we cannot assure you that an active trading market for our shares will continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for investors in our common stock to sell shares without depressing the market price for the shares or to sell the shares at all.

The trading price of the shares of our common stock has been and is likely to continue to be volatile.

Since our initial public offering, our stock price has been and is likely to continue to be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- the commencement, enrollment or results of the planned clinical trials of A-101 in patients with SK or any future clinical trials we may conduct, or changes in the development status of our drug candidates;

Table of Contents

- any delay in our regulatory filings for A-101 for the treatment of SK or any other drug candidate and any adverse development or perceived adverse development with respect to the applicable regulatory authority s review of such filings, including without limitation the FDA s issuance of a refusal to file letter or a request for additional information;
- adverse results from, delays in or termination of clinical trials;
- adverse regulatory decisions, including failure to receive regulatory approval of our drug candidates;
- unanticipated serious safety concerns related to the use of A-101 or any other drug candidate;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the market valuations of similar companies;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;
- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management s attention and resources from our business.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us or our business, our market and our competitors. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

The issuance of additional stock in connection with financings, acquisitions, investments, our equity incentive plan or otherwise will dilute all other stockholders.

Our certificate of incorporation authorizes us to issue up to 100,000,000 shares of common stock and up to 10,000,000 shares of preferred stock with such rights and preferences as may be determined by our board of directors. Subject to compliance with applicable rules and regulations, we may issue our shares of common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition, investment, our equity incentive plan or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

Table of Contents

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

Upon the closing of our initial public offering, the 5,750,000 shares sold in the offering became freely tradable and the remaining outstanding shares of common stock will be available for sale in the public market in April 2016 following the expiration of lock-up agreements between some of our stockholders and the underwriters. The representatives of the underwriters may release these stockholders from their lock-up agreements with the underwriters at any time and without notice, which would allow for earlier sales of shares in the public market.

In addition, we have filed a registration statement on Form S-8 under the Securities Act registering the issuance of approximately 3,900,000 shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under this registration statement on Form S-8 are available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and the restrictions of Rule 144 under the Securities Act in the case of our affiliates.

Additionally, the holders of an aggregate of 11,677,076 shares of our common stock, or their transferees, have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

There are provisions in our certificate of incorporation and bylaws that may make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change of control was considered favorable by some or all of our stockholders. For example, our board of directors has the authority to issue up to 10,000,000 shares of preferred stock. The board of directors can fix the price, rights, preferences, privileges, and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change of control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors is elected each year;

- stockholders are not entitled to remove directors other than by a 662/3% vote and only for cause;
- stockholders are not permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change of control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates beneficially own a majority of our common stock. As a result, these persons, acting together, would be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions. The interests of this group of stockholders may not coincide with our interests or the interests of other stockholders.

Table of Contents

We are an emerging growth company and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced Management s Discussion and Analysis of Financial Condition and Results of Operations disclosure in this report;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will 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. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) December 31, 2020, (2) the last day of the fiscal year in which we have total annual gross revenue of at least $1.0 billion, (3) the last day of the fiscal year in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th and (4) any date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards 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.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. Commencing with our fiscal year ending December 31, 2016, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our initial public offering, we had never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system s objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial

Table of Contents

statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC, or other regulatory authorities.

We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.

As of December 31, 2014, we had federal and state net operating loss carryforwards of $13.8 million and $13.8 million, respectively, and federal research and development tax credit carryforwards of $0.2 million, each of which if not utilized will begin to expire in 2032. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an ownership change, which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation s ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We have not determined if we have experienced Section 382 ownership changes in the past and if a portion of our net operating loss and tax credit carryforwards are subject to an annual limitation under Section 382. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If we determine that an ownership change has occurred and our ability to use our historical net operating loss and tax credit carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

We have broad discretion in the use of proceeds from our recent initial public offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We have broad discretion over the use of proceeds from our recent initial public offering. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. We expect to use the net proceeds from the offering to fund our research and development expenses and for working capital and general corporate purposes. Our failure to apply the net proceeds effectively could compromise our ability to pursue our strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. Stockholders will not have the opportunity to influence our decisions on how to use the net proceeds from the initial public offering.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future and our stock may not appreciate in value.

We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any existing or future debt agreements may preclude us from paying dividends. There is no guarantee that shares of our common stock will appreciate in value or that the price at which our stockholders have purchased their shares will be able to be maintained.

We will incur increased costs and demands upon management as a result of being a public company.

As a newly public company listed in the United States, we have begun, and will continue, particularly after we cease to be an emerging growth company, to incur significant additional legal, accounting and other costs. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and The NASDAQ Stock Market, may increase legal and financial compliance costs and make some activities more time-consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management s time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

Table of Contents

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim for breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

Table of Contents

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

(a) Sales of Unregistered Securities

From January 1, 2015 through September 30, 2015, we granted options to purchase an aggregate of 640,262 shares of our common stock under our 2012 equity compensation plan with an exercise price of $10.66 per share. The offers, sales and issuances of these options were exempt from registration under Rule 701 promulgated under the Securities Act, in that the transactions were under a written compensatory benefit plan as provided under Rule 701. The recipients of such securities were our employees, directors or consultants.

In August 2015, we issued an aggregate of 12,944,984 shares of our Series C convertible preferred stock to 28 investors at a purchase price of $3.09 per share, for aggregate consideration of $40.0 million. The offers, sales and issuances of these securities were exempt from registration under Section 4(a)(2) of the Securities Act and Regulation D promulgated under the Securities Act. Each of the purchasers represented to us that they acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the securities issued in these transactions. The purchasers also represented to us that they were accredited investors as defined in Rule 501 promulgated under the Securities Act.

On October 13, 2015, upon the closing of our initial public offering, all 40,286,041 shares of our then-outstanding convertible preferred stock were automatically converted into 11,677,076 shares of common stock. The issuance of such shares of common stock was exempt from the registration under Section 3(a)(9) of the Securities Act.

(b) Use of Proceeds from Public Offering of Common Stock

On October 6, 2015, our Registration Statement on Form S-1, as amended (File No. 333-206437) was declared effective in connection with our initial public offering, pursuant to which we sold 5,750,000 shares of our common stock, including the full exercise of the underwriters option to purchase additional shares, at a price to the public of $11.00 per share. The offering closed on October 13, 2015, and, as a result, we received net proceeds of $56.6 million (after underwriters discounts and commissions of $4.4 million and additional offering related costs of $2.2 million). The joint managing underwriters of the offering were Jefferies LLC and Citigroup Global Markets Inc.

No expenses incurred by us in connection with our initial public offering were paid directly or indirectly to (i) any of our officers or directors or their associates, (ii) any persons owning 10% or more of any class of our equity securities, or (iii) any of our affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board or board committee service.

There has been no material change in the planned use of proceeds from our initial public offering from that described in the final prospectus filed by us with the Securities and Exchange Commission on October 8, 2015 pursuant to Rule 424(b) of the Securities Act.

Item 6. Exhibits

Exhibit No.	Document
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated herein by reference to Exhibit 3.1 to the Registrant s Current Report on Form 8-K (File No. 001-37581), filed with the Commission on October 13, 2015).
3.2	Amended and Restated Bylaws of the Registrant (incorporated herein by reference to Exhibit 3.2 to the Registrant s Current Report on Form 8-K (File No. 001-37581), filed with the Commission on October 13, 2015).
4.1	Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to Exhibit 4.1 to Amendment No. 2 to the Registrant s Registration Statement on Form S-1 (File No. 333-206437), filed with the Commission on September 25, 2015).
10.1*	Amended and Restated Employment Agreement, by and between the Registrant and Neal Walker, dated as of October 5, 2015.
10.2*	Employment Agreement, by and between the Registrant and Stuart Shanler, dated as of October 4, 2015.
10.3*	Employment Agreement, by and between the Registrant and Christopher Powala, dated as of September 17,

Table of Contents

Exhibit No.	Document
	2015.
31.1*	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act.
31.2*	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act.
32.1**	Certifications of Principal Executive Officer and Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act.

* Filed herewith.

** These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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.

ACLARIS THERAPEUTICS, INC.

Date: November 18, 2015	By:	/s/ Neal Walker Neal Walker President and Chief Executive Officer *(On behalf of the Registrant)*
Date: November 18, 2015	By:	/s/ Frank Ruffo Frank Ruffo Chief Financial Officer *(Principal Financial Officer)*

Table of Contents

Exhibit Index

Exhibit No.	Document
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated herein by reference to Exhibit 3.1 to the Registrant s Current Report on Form 8-K (File No. 001-37581), filed with the Commission on October 13, 2015).
3.2	Amended and Restated Bylaws of the Registrant (incorporated herein by reference to Exhibit 3.2 to the Registrant s Current Report on Form 8-K (File No. 001-37581), filed with the Commission on October 13, 2015).
4.1	Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to Exhibit 4.1 to Amendment No. 2 to the Registrant s Registration Statement on Form S-1 (File No. 333-206437), filed with the Commission on September 25, 2015).
10.1*	Amended and Restated Employment Agreement, by and between the Registrant and Neal Walker, dated as of October 5, 2015.
10.2*	Employment Agreement, by and between the Registrant and Stuart Shanler, dated as of October 4, 2015.
10.3*	Employment Agreement, by and between the Registrant and Christopher Powala, dated as of September 17, 2015.
31.1*	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act.
31.2*	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act.
32.1**	Certifications of Principal Executive Officer and Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act.

* Filed herewith.

** These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.