Aclaris Therapeutics, Inc.
Form 10-K
March 23, 2016
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF

THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2015 Commission file number 001-37581

ACLARIS THERAPEUTICS, INC.

Incorporated under the Laws of the State of Delaware	I.R.S. Employer Identification No. 46-0571712

101 Lindenwood Drive, Suite 400

Malvern, PA 19355

(484) 324-7933

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

Title of Each Class:	Name of Each Exchange on which Registered
Common Stock, $0.00001 par value	The NASDAQ Stock Market, LLC

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

None

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

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

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

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

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

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

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

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

There was no aggregate market value of shares of common stock held by non-affiliates of the registrant as of June 30, 2015, the last business day of the registrant's most recently completed second fiscal quarter, because the registrant's common stock was not trading on any exchange on that date.

As of March 23, 2016, 20,157,503 shares of common stock, $0.00001 par value, were outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Company's definitive proxy statement, to be filed pursuant to Regulation 14A under the Securities Exchange Act of 1934, for its 2016 Annual Meeting of Stockholders are incorporated by reference in Part III of this Form 10-K.

Table of Contents

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K (this "Annual Report") contains 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, or the Exchange Act, that involve substantial risks and uncertainties. The forward-looking statements are contained principally in Part I, Item 1. "Business," Part I, Item 1A. "Risk Factors," and Part II, Item 7. "Management's Discussion and Analysis of Financial Condition and Results of Operations," but are also contained elsewhere in this Annual Report. In some cases, you can identify forward-looking statements by the words "may," "might," "will," "could," "would," "should," "expect," "intend," "plan," "objective," "anticipate," "believe," "estimate," "predict," "project," "potential," "ongoing," or the negative of these terms, or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report, we caution you that these statements are based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain. Forward-looking statements include statements about:

- our plans to develop and commercialize our drug candidates;
- the timing of our planned clinical trials of A-101 in patients with SK and our other drug candidates;
- the timing of our NDA filing for A-101 for the treatment of SK;
- the timing of and our ability to obtain and maintain regulatory approvals for our drug candidates;
- the clinical utility of our drug candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations about the willingness of patients to pay out of pocket for procedures using our drug candidates for the treatment of SK;
- our expectations about the willingness of dermatologists to use A-101 for the treatment of SK;
- our intellectual property position;
- our plans to in-license or acquire additional drug candidates for other dermatological conditions to build a fully integrated dermatology company; and
- our estimates regarding future revenue, expenses and needs for additional financing.

You should refer to "Item 1A. Risk Factors" in this Annual Report for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward looking statements. As a result of these factors, we cannot assure you that the forward looking statements in this Annual Report will prove to be accurate. Furthermore, if our forward looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. The forward-looking statements in this Annual Report represent our views as of the date of this Annual Report. We anticipate that subsequent events and developments may cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we undertake no obligation to publicly update any forward looking statements, whether as a result of new information, future events or otherwise, except as required by law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Annual Report.

2

Table of Contents

TABLE OF CONTENTS

Table of Contents

PART I

Item 1. Business

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 Topical Solution, 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 statistically significant improvements in clearing SK lesions on the face, trunk and extremities of the body. In the first quarter of 2016, we initiated two multi-center, double blind Phase 3 clinical trials and one open label Phase 3 clinical trial of A-101 in patients with SK. If the results of these trials are favorable, we plan 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, also known as verruca vulgaris, and A-102, a proprietary gel dosage form of hydrogen peroxide, as a prescription treatment for SK and common warts. In the fourth quarter of 2015, we initiated a Phase 2 clinical trial to evaluate A-101 for the treatment of common warts. We have also 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 and technologies to build a fully integrated dermatology company.

SK lesions are among the most common non-malignant skin tumors and one of the most frequent diagnoses made by dermatologists. SK lesions typically have a waxy, scaly, slightly elevated appearance, and multiple lesions are often present. Though the lesions are non-malignant, patients often elect to have their condition treated by a dermatologist, either because the lesions have become inflamed or because the patient feels they are cosmetically unattractive. SK lesions are usually treated by cryosurgery, electrodesiccation, curettage or excision. Each of these methods may be painful or can result in pigmentary changes or scarring at the treatment site. No drugs have been approved by the FDA for the treatment of SK.

A study published in the Journal of The American Academy of Dermatology in 2006, which we refer to as the AAD study, estimated that SK affects over 83 million people in the United States. Based on a market survey we commissioned in 2014, we estimate that there are 18.5 million patient visits to dermatologists for SK and dermatologists perform approximately 8.3 million procedures to remove SK lesions annually in the United States. We estimate that the cost of these procedures to third-party payors and patients is more than $1.2 billion annually.

In June 2014, we completed our Phase 2 clinical trial of A-101 in 35 patients with four SK lesions on the trunk; in December 2014, we completed our Phase 2 clinical trial of A-101 in 172 patients with four SK lesions on the trunk and extremities; and in March 2015, we completed our Phase 2 clinical trial of A-101 in 119 patients with a single SK lesion on the face. In each of these trials, following one or two applications of the two highest concentrations of A-101, we observed clinically relevant and statistically significant improvements in clearing SK lesions.

In the first quarter of 2016, we initiated three Phase 3 clinical trials of A-101 in patients with SK lesions on the face, trunk and extremities. Two of these trials are being conducted on a double-blind basis, while the third is open label. If the results of these Phase 3 clinical trials are favorable, we intend to submit our NDA for A-101 for the treatment of SK to the FDA in the fourth quarter of 2016 and build a specialty sales force to market the product to dermatologists in the United States. We plan to seek a collaborator to commercialize A-101, if approved, in the European Union. We have the exclusive right to commercialize A-101, if approved, in various countries throughout the world.

We also plan to develop A-101 for the treatment of common warts. Although common warts are generally not harmful and in most cases eventually clear without any medical treatment, they may be painful and aesthetically unattractive and are contagious. On an annual basis, 1.9 million people are diagnosed with common warts. The AAD study estimated that annual direct expenditures for patients seeking treatment for warts of all types in a medical office were $939 million, including the cost of the office visit as well as the treatments. We estimate that approximately one-half of those expenditures were for the treatment of common warts. Common warts can be removed with slow-acting,

Table of Contents

over-the-counter products containing salicylic acid. As with SK, cryosurgery is the most frequently used in-office treatment for common warts. No prescription drugs have been approved by the FDA for the treatment of common warts. We completed toxicology studies and commenced a Phase 2 clinical trial of A-101 for the treatment of common warts in the fourth quarter of 2015. In addition to A-101, we are also developing A-102, a proprietary topical gel dosage form of hydrogen peroxide, for the treatment of both SK and common warts.

In addition, we plan to develop the JAK inhibitors, A-201 and A-301, which we in-licensed from Rigel Pharmaceuticals, Inc., or Rigel, as potential treatments for AA. AA is an autoimmune dermatologic condition typically characterized by patchy non-scarring hair loss on the scalp and body. More severe forms of AA include total scalp hair loss, known as alopecia totalis, and total hair loss on the scalp and body, known as alopecia universalis. AA affects up to 0.2% of people globally, with two-thirds of affected individuals being 30 years old or younger at the time of disease onset. Treatment options for the less severe, patchy forms of AA include corticosteroids, either topically applied or injected directly into the scalp where the bare patches are located, or the induction of an allergic reaction at the site of hair loss using a topical contact sensitizing agent, an approach known as topical immunotherapy. The same treatment options are utilized for the more severe forms of AA, although utilization of these treatment options for the more severe forms of AA is limited due to limited efficacy, certain side effects, and their impracticality for extensive surface areas. We plan to develop A-201 as an oral treatment for alopecia totalis and alopecia universalis and A-301 as a topical treatment for patchy AA. We plan to submit an investigational new drug application, or IND, in the second half of 2016 for A-201 and commence clinical trials in the first half of 2017. For A-301, we plan to submit an IND and commence clinical trials in the first half of 2017.

Our intellectual property portfolio contains issued patents directed to methods of use for A-101 and our lead JAK inhibitors, A-201 and A-301. With respect to A-101, our issued patents begin to expire in 2022, subject to any applicable patent term extension that may be available in a particular country. Our intellectual property portfolio also contains a U.S. and a Patent Cooperation Treaty, or PCT, patent application directed to, among other things, formulations and methods of use for A-101 and a single-use, self-contained, pre-filled, disposable pen-type applicator for use with such formulations, including A-101. Our pending U.S. and PCT patent applications, if they issue as patents, would be expected to expire in 2035, subject to any applicable patent term adjustment or extension that may be available in a particular country. With respect to our JAK inhibitor drug candidates, the issued U.S. and foreign patents expire between 2023 and 2030, subject to any applicable patent term extension that may be available in a particular country. Our intellectual property portfolio also contains pending applications directed to, among other things, the use of JAK inhibitors that, if issued as patents, would be expected to expire in 2034, subject to any applicable patent term adjustment or extension that may be available in a particular country.

Table of Contents

Our Drug Candidates

We have utilized our experience to establish a pipeline of drug candidates that we believe will address significant unmet needs in dermatology. Our pipeline of drug candidates is summarized in the table below:

Our Lead Drug Candidate: A-101 for the Treatment of Seborrheic Keratosis

Overview

We are developing A-101 for the treatment of SK. SK lesions typically have a waxy, scaly, slightly elevated appearance, and multiple lesions are often present. The lesions can vary in color from light tan to dark brown or black and typically appear on the face, trunk and extremities. Though the lesions are non-malignant, patients often elect to have their condition treated by a dermatologist, either because the lesions have become inflamed or because the patient feels they are cosmetically unattractive.

Table of Contents

We have completed three Phase 2 clinical trials in over 300 patients with SK and observed clinically relevant and statistically significant improvements in clearing SK lesions on the face, trunk and extremities of the body following one or two applications of A-101. The following table summarizes the design of these clinical trials:

Name of Clinical Trial and Number of Subjects Enrolled	SK Lesion Area	Date Completed	Trial Design	Trial Objective
SEBK-203 (n=119)	Face	March 2015	Multi-center, randomized, double-blind, vehicle-controlled, parallel group One lesion treated A-101 concentrations: 32.5%, 40.0% Duration: 106 days	Evaluate safety, efficacy, tolerability and dose-response profile of two concentrations of A-101 vs. vehicle control
SEBK-202 (n=172)	Trunk and Extremities	December 2014	Multi-center, randomized, double-blind, vehicle-controlled, parallel group Four lesions treated A-101 concentrations: 32.5%, 40.0% Duration: 106 days	Evaluate safety, efficacy, tolerability and dose-response profile of two concentrations of A-101 vs. vehicle control
SEBK-201 (n=35)	Trunk (Back)	June 2014	Double-blind, vehicle-controlled intra-subject Four lesions treated A-101 concentrations: 25.0%, 32.5%, 40.0% Duration: 78 days	Evaluate safety, efficacy and tolerability of three concentrations of A-101 vs. vehicle control

A-101 Mechanism of Action

SK is a slowly growing epidermal tumor consisting of an abnormal accumulation of hyper-adherent senescent cells exhibiting decreased cell death. Senescent cells are no longer capable of dividing but are still alive and metabolically active. SK lesions may be amenable to a topically delivered agent that could both break down the abnormal intercellular connections between the cells and promote death of the abnormal SK cells.

Hydrogen peroxide is a potent and important oxidizing agent in the human body. Local concentrations of hydrogen peroxide are carefully controlled by a complex antioxidant defense system consisting of both enzymes and nonenzymatic components. The topical application of high concentrations of hydrogen peroxide to SK lesions can locally overwhelm this antioxidant defense system in the skin, allowing hydrogen peroxide to penetrate the surface of the lesion, react with the abnormal SK cells, and remove or dissolve the SK lesions.

Through a process known as lipid peroxidation, free radical molecules generated by hydrogen peroxide degrade the phospholipids of the cell membrane, leading to the breakdown, or lysis, of the lipid membrane of the cell. This chemical reaction is followed by the denaturation, or loss of structure, of proteins within the cell, as well as oxidative DNA and mitochondrial damage. This series of events induces cell death of abnormal SK cells, either through the process of programmed cell death, known as apoptosis, or through cell injury, known as necrosis.

Table of Contents

The following graphic illustrates this mechanism of action for A-101:

Response of Seborrheic Keratosis Cells to A-101

Clinical Development

We submitted an IND for A-101 for the treatment of SK to the FDA in September 2013 and have completed three Phase 2 clinical trials under this IND. In February 2015, we held a Type C meeting with the FDA at which we discussed clinical endpoints to support a claim of efficacy, as well as the statistical methodology we plan to use in our Phase 3 clinical trials. In May 2015, we held an end-of-Phase 2 meeting with the FDA to discuss our A-101 development program leading to a potential NDA submission.

Phase 2 Clinical Trial of A-101 in Subjects with Seborrheic Keratosis on the Face (SEBK-203)

Trial Design

We commenced a Phase 2 clinical trial in October 2014 that was a multi-center, randomized, double-blind, vehicle-controlled, parallel group trial designed to evaluate the safety, tolerability, initial efficacy and dose-response profile of A-101 topical solution at 32.5% and 40.0% concentrations and a topical solution vehicle control. We completed the trial in March 2015. We enrolled 119 subjects in the trial at four sites in the United States, and 116 subjects completed the trial. Three of the 119 subjects withdrew from the trial due to unrelated adverse events. Of the 116 subjects who completed the trial, 37 subjects received the 40.0% concentration, 39 subjects received the 32.5% concentration and 40 subjects received the vehicle control. The age of the subjects ranged from 33 to 93, with a mean age of 70. Of the 116 subjects who completed the trial, 53 were male, 63 were female and all were Caucasian, with a variety of skin types. Inclusion criteria included a clinical diagnosis of stable, clinically typical SK and one appropriate SK target lesion on the subject's face of specified size and thickness. Exclusion criteria included clinically atypical or rapidly growing SK lesions and the use of specified topical or systemic therapies within a defined time period prior to the first visit.

The evaluation period consisted of 15 weeks after initial treatment. At the first visit, the investigator identified a single target lesion on the face of each subject for treatment. During the second visit, or baseline, which occurred on Day 1 of the evaluation period, eligible subjects were randomized to receive the vehicle control or one of the two active

concentrations of A-101 and the applications were performed by non-physician staff. No applications were made at a visit on Day 8. At Day 22, any target lesion that met the retreatment criteria received a second application of the assigned concentration of A-101 or vehicle control. The subjects were evaluated at multiple visits through Day 106, but no applications were made after Day 22.

Table of Contents

Endpoints

The primary endpoint of this clinical trial was the mean change from baseline in the Physician's Lesion Assessment, or PLA, score at the end of the trial. The PLA score is a method we have developed and validated to measure the severity of lesions and uses a scale ranging from zero to three. Secondary endpoints included responder analysis of PLA scores of zero or one. In this trial, a PLA score of zero represented no visible lesion; a PLA score of one represented near clearance, meaning a visible lesion that, while not elevated, has a surface appearance that is different from the surrounding skin; a PLA score of two represented a visible lesion that is elevated but with a thickness of less than or equal to one millimeter; and a PLA score of three represented a visible lesion with a thickness exceeding one millimeter.

Efficacy Results

As shown in the table below, for the primary endpoint, mean change from baseline in PLA score, we observed statistically significant improvements as compared to the vehicle for both concentrations of A-101 evaluated, with the 40.0% concentration being the most effective. The results for the active treatment groups were statistically significant with a p-value of less than 0.001. P-value is a conventional statistical method for measuring the statistical significance of clinical results. A p-value of less than 0.05 is generally considered to represent statistical significance, meaning that there is a less than five percent likelihood that the observed results occurred by chance.

Mean Change from Baseline in PLA Score — Face

In addition, we measured the percentage of subjects who achieved total clearance, or a PLA of zero, at Day 22 and Day 106. These results are presented in the table below. At Day 22, 24.0% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance and 28.0% of the subjects receiving A-101 at the 32.5% concentration achieved total clearance, compared to none in the vehicle control group. At Day 106, 60.0% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance and 46.0% of subjects receiving A-101 at the 32.5% concentration achieved total clearance, compared to 3.0% in the vehicle control group. These results were statistically significant, with a p-value of less than 0.001.

Table of Contents

Percentage of Subjects with Clear Lesions — Face

We also measured the percentage of subjects who achieved either total clearance or near clearance, or a PLA score of either zero or one, at Day 22 and Day 106. These results are presented in the table below. At Day 22, 45.9% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance or near clearance and 33.3% of the subjects receiving A-101 at the 32.5% concentration achieved total clearance or near clearance, compared to 2.5% in the vehicle control group. At Day 106, 67.6% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance or near clearance and 61.5% of subjects receiving A-101 at the 32.5% concentration achieved total clearance or near clearance, compared to 5.0% in the vehicle control group. These results were statistically significant, with a p-value of less than 0.001.

Percentage of Subjects with Clear or Near-Clear Target Lesions — Face

Table of Contents

Safety Results

A-101 was generally well tolerated at both the 32.5% and 40.0% concentrations. While two subjects in each of the 32.5% and 40.0% concentration treatment groups reported severe stinging after administration, most local skin reactions were considered to be transient and mild or moderate. Treatment-emergent adverse events were reported by 29 subjects. However, only one of these adverse events, slight bleeding at the sight of administration, was determined by the investigator to be drug-related. Four subjects reported serious adverse events, but none were considered to be related to treatment by the investigator. Three subjects dropped out of the trial due to adverse events unrelated to treatment.

Phase 2 Clinical Trial of A-101 in Subjects with Seborrheic Keratosis on the Trunk and Extremities (SEBK-202)

Trial Design

In June 2014, we commenced a Phase 2 clinical trial that was a multi-center, randomized, double-blind, vehicle-controlled, parallel group trial designed to evaluate the safety, tolerability, initial efficacy and dose-response profile of A-101 topical solution with concentrations of 32.5% and 40.0% and a topical solution vehicle control. We completed the trial in December 2014. We enrolled 172 subjects in the trial at five sites in the United States, and 169 subjects completed the trial. Of the 172 subjects enrolled in the trial, 57 subjects received the 40.0% concentration, 57 subjects received the 32.5% concentration and 58 subjects received the vehicle control. Of the three subjects who withdrew from the trial, one subject withdrew due to inconvenience, one subject moved and one subject withdrew due to lack of follow-up by the investigator. The age of the subjects ranged from 48 to 97, with a mean age of 69. Of the 172 subjects enrolled in the trial, 91 were male, 81 were female and all but two were Caucasian. There were a variety of skin types within the trial population. Inclusion criteria included a clinical diagnosis of stable, clinically typical SK and at least four SK target lesions on the subject's trunk, defined as the upper body excluding the head and limbs, or extremities with a PLA of at least 2.0 and of specified size and thickness. Exclusion criteria included clinically atypical or rapidly growing SK lesions and the use of specified topical or systemic therapies within a defined time period prior to the first visit.

The evaluation period consisted of 15 weeks after initial treatment. At the first visit, the investigator identified four target lesions on the trunk or extremities of each subject for treatment. During the second visit, or baseline, which occurred on Day 1 of the evaluation period, eligible subjects were randomized to receive the vehicle control or one of the two active concentrations of A-101 and the applications were performed by non-physician staff. No applications were made at a visit on Day 8. At Day 22, any target lesion that met the retreatment criteria received a second application of the assigned concentration of A-101 or vehicle control. The subjects were then evaluated at multiple visits through Day 106, but no applications were made after Day 22.

Endpoints

The primary endpoint of this clinical trial was the percentage of the four target SK lesions judged to be clear, meaning a PLA of zero, for each patient at the end of the trial. Secondary endpoints included the change from baseline PLA. In this trial, we used the same PLA score we used in our trial in subjects with SK lesions on the face (SEBK-203).

Efficacy Results

As shown in the table below, for the primary endpoint, the mean percentage of the four target SK lesions that were judged to be cleared for each patient at Day 106, we observed clinically relevant and statistically significant improvement for both concentrations of A-101 evaluated, with mean per-subject clearance of 26.8% and 45.1% at the

Table of Contents

32.5% and 40.0% concentrations, respectively, compared to only 4.8% mean per-subject clearance in the vehicle control group. The results for the active treatment groups were statistically significant with a p-value of less than 0.001.

Mean Per-Subject Percentage Clearance — Trunk and Extremities

We also measured the percentage of subjects who achieved total clearance, or a PLA score of zero, in all four of their lesions. These results are presented in the table below. Of the subjects receiving A-101 with 40.0% and 32.5% concentrations, 19.6% and 16.1%, respectively, had clearance of all lesions at Day 106, compared to none in the vehicle control group. These results were statistically significant, with a p-value of less than 0.01. At Day 22, 14.3% of the subjects receiving A-101 at the 40.0% concentration had achieved clearance of all lesions, a result that was also statistically significant, with a p-value of less than 0.01. Only 5.4% of subjects receiving A-101 at the 32.5% concentration achieved clearance of all lesions at Day 22, compared to none in the vehicle control group, but this result for the 32.5% group was not statistically significant.

Percentage of Subjects Achieving Total Clearance — Trunk and Extremities

We also measured the percentage of subjects who achieved either total clearance or near clearance, or a PLA score of either zero or one, in all four of their lesions. These results are presented in the table below. At Day 22, 32.6% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance or near clearance and 23.7% of the subjects receiving A-101 at the 32.5% concentration achieved total clearance or near clearance, compared to 1.8% in the vehicle control group. At Day 106, 58.9% of the subjects receiving A-101 at the 40.0% concentration achieved total clearance or near clearance and 42.4% of the subjects receiving A-101 at the 32.5% concentration achieved total

Table of Contents

clearance or near clearance, compared to 10.1% in the vehicle control group. These results were statistically significant with a p-value of less than 0.0001.

Percentage of Subjects Achieving Total Clearance or Near Clearance — Trunk and Extremities

Safety Results

A-101 was generally well tolerated at both the 32.5% and 40.0% concentrations. Local skin reactions were treatment- and dose-related, and most were considered to be transient and mild to moderate. Treatment-emergent adverse events were reported by 45 subjects. Only one of these events, moderate tenderness at a treatment site on the subject's thigh, was determined by the investigator to be drug-related. Three subjects reported serious adverse events, but none were considered to be related to treatment by the investigator. None of the subjects dropped out of the trial due to adverse events.

Phase 2 Clinical Trial of A-101 in Subjects with Seborrheic Keratosis on the Trunk (Back) (SEBK-201)

Trial Design

We commenced a Phase 2 clinical trial of A-101 in November 2013 that was a double-blind, vehicle-controlled intra-subject clinical trial designed to evaluate the safety, tolerability and initial efficacy of A-101 in clearing SK lesions. The trial compared three active concentrations of A-101, 40.0%, 32.5% and 25.0%, with a vehicle solution control. In the trial, each subject received each of the four treatments on four separate lesions on the back. We enrolled 35 adult subjects in the trial at one site in the United States. We completed the trial in June 2014. Of the 35 subjects enrolled in the trial, one subject withdrew from participation in the trial due to the distance between the subject's home and the clinical trial site. The age of the subjects ranged from 55 to 85, with a mean of 69 years. Of the 35 subjects enrolled in the trial, 20 of the subjects were female and 15 were male, and all subjects were Caucasian. Inclusion criteria included a clinical diagnosis of stable clinically typical SK and at least four appropriate SK target lesions on the subject's back. Exclusion criteria included clinically atypical or rapidly growing SK lesions and the use of specified topical or systemic therapies within a defined time period prior to the first visit.

Table of Contents

The evaluation period consisted of 11 weeks after initial treatment. At the first visit, the investigator identified four target lesions on the back for treatment. During a second visit, or baseline, which occurred on Day 1 of the evaluation period, lesions on each subject were randomized to receive the vehicle control or one of the three active concentrations of A-101, and the applications were performed by non-physician staff. No applications were made at visits on Day 8 and Day 15. On Day 22, any target lesion that met the retreatment criteria received a second application of the assigned concentration of A-101 or vehicle control. No applications were made at subsequent visits, which occurred on Days 29, 43, 57 and 78.

Endpoints

The primary endpoint of this clinical trial was reduction in PLA score from baseline over a period of 78 days, as well as the physician's subjective assessment of the condition of the lesion. In this trial, we used an earlier version of the PLA scale in which a PLA score of zero was considered to be complete clearance of the lesion, a PLA score of one represented the lesion was barely evident on examination, a PLA score of two represented an obvious lesion, while a PLA score of three represented a severe, prominent lesion. This PLA scale was subsequently refined in our later trials to make it more clinically objective.

Efficacy Results

For the 34 subjects that completed the trial, the efficacy results are presented in the table below. We measured the proportion of PLA complete responders, defined as a PLA score of zero at Day 78, in each treatment group. Of the 34 lesions treated with the 40.0% concentration, 11 lesions, or 32.4%, completely responded, a result that was statistically significant with a p-value of less than 0.001. Of the 34 lesions treated with the 32.5% concentration, 5 lesions, or 14.7%, completely responded, a result that was statistically significant with a p-value of less than 0.05. Of the 34 lesions treated with the 25.0% concentration, 3 lesions, or 8.8%, completely responded, a result that was not statistically significant. There were no complete responders in the vehicle control group.

Percentage of Complete Responders — Trunk (Back)

Table of Contents

We also measured the proportion of PLA complete responders or near complete responders, defined as a PLA score of zero or one, at Day 78, in each treatment group. These results are presented in the table below. Of the 34 lesions treated with the 40.0% concentration, 23 lesions, or 67.7%, were complete or near complete responders, a result that was statistically significant with a p-value of less than 0.0001. Of the 34 lesions treated with the 32.5% concentration, 19 lesions, or 55.9%, were complete or near complete responders, a result that was statistically significant with a p-value of less than 0.0001. Of the 34 lesions treated with the 25.0% concentration, 12 lesions, or 35.3%, were complete or near complete responders, a result that was statistically significant with a p-value of less than 0.05. Four lesions, or 11.8%, of the lesions treated with vehicle control either were complete or near complete responders.

Percentage of Complete or Near Complete Responders — Trunk (Back)

Safety Results

A-101 was generally well tolerated at the 25.0%, 32.5% and 40.0% concentrations. Local skin reactions were transient and treatment- and dose-related, and most were considered to be mild to moderate. Treatment-emergent adverse events were reported by nine subjects, and none of those reported were considered to be treatment-related. The only treatment-emergent adverse events reported by more than one subject were seasonal allergy in ten subjects and arthritis in four subjects. One subject had a serious adverse event of kidney infection, which was considered by the investigator to be unrelated to treatment. None of the subjects dropped out of the trial due to an adverse event and no adverse event led to trial discontinuation.

Phase 3 Clinical Program

In the first quarter of 2016, we initiated two multi-center, double-blind Phase 3 clinical trials and a third open-label Phase 3 clinical trial with subjects with SK lesions on the face, trunk and extremities.

We expect to enroll a total of approximately 1,000 subjects with SK on the face, trunk and extremities in these three trials, which are designed to evaluate the efficacy and safety of treatment with A-101 relative to vehicle. The first two clinical trials are randomized, multi-center, double-blinded, vehicle-controlled, parallel group Phase 3 clinical trials being conducted in the United States. We expect to enroll approximately 400 subjects with four SK lesions on the face, trunk and extremities in each of these two trials. In each of these two trials, subjects will be randomized to receive A-101 topical solution at the 40.0% concentration or vehicle on Day 1 and, if needed, on Day 22. In the third Phase 3 clinical trial, approximately 200 subjects with four SK lesions on the face, trunk and extremities will receive up to four treatments of A-101 twenty-one days apart on an open-label basis in order to gather additional safety data

on the extended use of A-101. The primary endpoint for the two multi-center, double-blind Phase 3 clinical trials will be the percentage of subjects who experience a complete clearance, meaning a PLA score of zero, for all four of the target SK lesions. The open label Phase 3 clinical trial will evaluate the safety of A-101 in subjects with four SK lesions on the face, trunk and extremities.

Table of Contents

We anticipate that our NDA and Marketing Authorization Application, or MAA, in the European Union for A-101 in SK will be based on the data collected from each of the three Phase 3 clinical trials. We believe that if these results are favorable, such results would be sufficient to support an NDA for the treatment of SK in the United States and may be sufficient to support a MAA for the treatment of SK in the European Union.

Additional Development Programs — A-101 for Common Warts

Ongoing Phase 2 Clinical Trial

Following a 12-week toxicology study in minipigs, in December 2015, we commenced a Phase 2 clinical trial of A-101 for the treatment of common warts. This double-blinded, randomized Phase 2 trial is being conducted at six sites in the United States and is designed to evaluate the safety, tolerability and dose-response of two concentrations of A-101 compared with a vehicle control consisting of placebo. We intend to enroll approximately 108 subjects in this clinical trial. Subjects will be randomized in three equal groups to receive either A-101 topical solution at the 40.0% or 45.0% concentrations or placebo. The primary endpoint for this clinical trial is the mean change in the Physician’s Wart Assessment (PWA) score at the end of the study. We expect to receive results from this clinical trial in the third quarter of 2016.

Investigator-Sponsored Trial

A trial was conducted by Dr. Steven Grekin, a dermatologist, using A-101 topical solution in subjects with common warts. This physician's IND for the treatment of common warts was submitted to the FDA in March 2014. This trial was a double-blind, vehicle-controlled trial comparing the 40.0% concentration of A-101 and a vehicle control. This trial was conducted at the Grekin Skin Institute in Michigan. In this trial, each subject received four treatments on one target wart. Twenty-two subjects were enrolled in the trial, with fifteen subjects completing the trial. Four subjects who were receiving vehicle control did not complete the trial because they were not satisfied with the results and three subjects who were receiving A-101 did not complete the trial for reasons unrelated to treatment. Of the subjects who completed the trial, nine subjects received the 40.0% concentration of A-101 and six subjects received the vehicle control. Subjects were at least 18 years old with a common wart on the hand.

We believe the results of the investigator-sponsored trial provided proof-of-concept data for the treatment of common warts with A-101. Efficacy measures were evaluated at week 6, two weeks after the last treatment. The trial evaluated the mean change from baseline using a wart severity assessment scale ranging from zero to three. A wart severity assessment score of zero means the subject has no clinically diagnosable wart, a score of one means the subject has a barely evident clinically diagnosable wart, a score of two represents an obvious wart and a score of three represents a conspicuous wart. All of the subjects enrolled in this trial had a wart severity assessment score of at least two. The wart severity assessment score results are presented in the table below. The data from the trial showed statistically

significant improvements in subjects treated with A-101 compared to vehicle control in the mean wart severity assessment score.

Mean Change from Baseline in Wart Severity Assessment Score

Table of Contents

The trial also evaluated the mean wart improvement assessment score in subjects. The wart improvement assessment scale measures the level of improvement and ranges from zero to five. A wart improvement assessment score of zero means the common wart is completely cleared, a score of one means the wart markedly improved compared to baseline, a score of two means the wart moderately improved compared to baseline, a score of three means the wart mildly improved compared to baseline, a score of four means there was no change and a score of five means the wart worsened compared to baseline. The mean wart improvement assessment score results are presented in the table below. The data from the trial showed statistically significant improvements in subjects treated with A-101 compared to vehicle control in the mean wart improvement assessment score.

Mean Wart Improvement Assessment Score

A-101 was well tolerated in these subjects with no adverse events reported.

Additional Development Programs — A-201 and A-301 for Alopecia Areata

Overview

We plan to develop A-201 and A-301 as potential treatments for AA. AA is an autoimmune dermatologic condition, typically characterized by patchy, non-scarring hair loss on the scalp and body. More severe forms of AA include alopecia totalis and alopecia universalis. Treatment options for the less severe, patchy forms of AA include corticosteroids, either topically applied or injected directly into the scalp where the bare patches are located, and the induction of an allergic reaction at the site of hair loss using a topical contact sensitizing agent, an approach known as topical immunotherapy. The same treatment options are utilized for the more severe forms of AA, although utilization of these treatment options for the more severe forms of AA is limited due to limited efficacy, side effects, and their impracticality for use on extensive surface areas. We plan to develop A-201 as an oral treatment for alopecia totalis and alopecia universalis and A-301 as a topical treatment for patchy AA. For A-201, we plan to submit an IND in the second half of 2016 and commence clinical trials in the first half of 2017. For A-301, we plan to submit an IND and commence clinical trials in the first half of 2017.

A-201 and A-301 Mechanism of Action

Though the exact cause of AA remains unclear, clinical and physiological evidence suggests that the primary pathologic process of AA is a T-cell mediated autoimmune attack on the hair follicles.

Cytokines are proteins that bind to cell surface receptors and initiate a signaling process that ultimately leads to modulation of gene expression. The JAK family of enzymes plays an essential role in regulating the signaling process of most cytokines in cells by linking cytokine signaling from the cell surface membrane receptors to signal transducers and activators of transcription, or STATs, within the cells. The binding of a cytokine to the appropriate receptor on the cell surface results in the activation of the JAK protein, which in turn activates the STATs.

Table of Contents

The JAK proteins are essential for modulating many immunological and inflammatory processes, and, in conditions characterized by an abnormally upregulated immune response, JAK inhibitors have been found to be effective in downregulating the abnormally activated JAK-STAT pathway and alleviating manifestations of disease.

Most recently, it has been reported that systemically administered JAK inhibitors may be potentially efficacious in the treatment of AA, both in its patchy and more severe forms. In a mouse model of AA, systemically administered JAK inhibitors prevented the development of AA, and topically administered JAK inhibitors promoted hair regrowth. In a clinical trial evaluating ruxolitinib, an oral JAK inhibitor, as a potential treatment for cancer, three human patients with moderate-to-severe AA treated with ruxolitinib achieved near-complete hair regrowth within three to five months of treatment. In another clinical trial evaluating baricitinib, an oral JAK inhibitor, for the treatment of a rare autoinflammatory disorder called CANDLE syndrome, a patient with concomitant AA exhibited complete resolution of his hair loss after nine months of therapy.

Manufacturing

We do not have any manufacturing facilities or personnel. We rely on third parties for the manufacture of A-101 for preclinical studies and clinical trials, and will continue to rely on third parties for the commercial manufacture of A-101 if it receives marketing approval. For hydrogen peroxide, the active pharmaceutical ingredient, or API, in A-101, we have entered into an exclusive, ten-year, automatically renewable supply agreement with PeroxyChem LLC, or PeroxyChem, a manufacturer of hydrogen peroxide, to provide the API that can be used in A-101 for the treatment of SK and a number of other specified dermatological indications. We or PeroxyChem may terminate the supply agreement with prior written notice immediately for specified financial reasons, after a 10-day and 60-day cure period for material monetary and non-monetary material breaches, respectively, and in the event of a force majeure event, including if the FDA does not approve A-101 for commercial sale in the United States, that continues for 90 consecutive days. In addition, we may terminate the PeroxyChem supply agreement, with prior written notice, for PeroxyChem's failure to supply API to us for more than 90 cumulative days in a year.

For some of the components used in connection with the manufacture and assembly of the pen-type applicator for A-101, we purchase our components from third-party manufacturers on a purchase order basis and do not have supply arrangements in place. In addition, we have engaged third parties for the supply and assembly of components of the pen-type applicator and the assembly, labeling and packaging of the finished drug product to be used in our planned Phase 3 clinical trials and for commercial purposes, if A-101 is approved for marketing.

Replacement of any of these third-party manufacturers would require us to qualify new manufacturers and negotiate and execute contractual agreements with them. If any of our supply or service agreements with third-party manufacturers are terminated, we will experience delays and additional expenses in the completion of the development of and obtaining regulatory approval for our lead drug candidate, A-101 for the treatment of SK.

Commercialization

For A-101, we expect to retain U.S. commercial rights and to establish collaborations with third parties to commercialize A-101 outside the United States. We have not established any meaningful sales, marketing or product distribution operations to date because A-101 is still in clinical development. We plan to establish the required capabilities within an appropriate time frame ahead of any potential drug approval and commercialization in order to support a commercial product launch. If we commercialize A-101, or any other drug candidates that we may successfully develop, in the United States, we intend to build a targeted sales force to establish relationships with dermatologists. We believe a scientifically oriented, customer-focused team of approximately 50 to 60 sales representatives would allow us to reach the approximately 5,000 dermatologists in the United States with the highest potential for using A-101, who we estimate will continue to account for over 70% of in-office SK treatments performed. We expect that our sales force will be supported by sales and marketing management, internal sales and marketing support and commercial product distribution support.

We believe dermatologists will be inclined to adopt A-101 to treat their patients with SK, if it is approved, not only because of its clinical profile, but also because it may provide an expanded source of revenue for their practices. Dermatologists expect declining reimbursements from third-party payors for providing medical services. In addition, a greater portion of the cost of medical care has been shifted to patients, in the form of higher deductibles and co insurance. Collecting from patients can be difficult and costly for physician practices. We believe many

Table of Contents

dermatologists are interested in expanding the cash-pay aesthetic portion of their practices, meaning the portion of procedures that are not medically necessary and not reimbursed by third-party payors, by treating new aesthetic patients and by offering new services to current aesthetic patients. Though SK patients typically come into the dermatology practice seeking a medical diagnosis, we believe they often are willing to pay for removal of SK lesions to improve appearance even after they learn that the lesions are non-malignant and that removal may not be reimbursed. In addition, since A-101 can be administered by non-physician staff, we believe it could provide incremental practice revenue with minimal time commitment by the dermatologist after the diagnosis is made.

In 2014, there were approximately 10,000 dermatologists practicing in the United States. We believe dermatologists tend to be particularly focused on the safety of pharmaceutical products because, while skin diseases can have profound effects on patients' quality of life, few are life-threatening. As a result, we believe that dermatologists, as well as their patients, often prefer to use topical treatments when possible to limit the risk of systemic side effects. Dermatologists also tend to place a high level of emphasis on products that are easy to use because they often manage high volumes of patients. We believe this also contributes to a general preference for topical treatments. Finally, in our experience, dermatologists tend to engage with sales and medical affairs personnel from the pharmaceutical industry regarding the scientific evidence supporting dermatology products and the challenges experienced by physicians and patients in the use of these products. Dermatologists often rely on trusted relationships with scientifically oriented, customer-focused sales representatives who can provide them with the necessary information to support their use of appropriate treatments.

Competition

The pharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary drugs. While we believe that our knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical and specialty pharmaceutical companies, academic institutions and governmental agencies and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing treatments and new treatments that may become available in the future.

The key competitive factors affecting the success of A-101, if approved for the treatment of SK, are likely to be its efficacy, safety, non-invasiveness, pain profile and ability to be administered by non-physician staff. With respect to A-101 for the treatment of SK, we are aware of one biopharmaceutical company, BioLineRx Ltd., that is developing a combination drug candidate that targets SK, and another company, Skincential Sciences, Inc., that currently markets a line of cosmetic products targeting skin conditions, including SK. We are also aware of early research being conducted with Akt inhibitors as a potential treatment for SK. None of these products have been approved by the FDA for the treatment of SK in the United States.

With respect to A-101 for the treatment of common warts, we are aware of one company, Nielsen BioSciences, that is developing a prescription treatment for common warts. We are aware of another company, G&E Herbal

Biotechnology Co., LTD, 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 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

Table of Contents

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 subject registration for clinical trials, as well as in acquiring technologies complementary to, or that may be necessary for, our programs.

Intellectual Property

Our success depends in large part upon our ability to obtain and maintain proprietary protection for our drug candidates and to operate without infringing the proprietary rights of others. We seek to avoid the latter by monitoring patents and publications that may affect our business, and to the extent we identify such developments, evaluate and take appropriate courses of action. Our policy is to protect our proprietary position by, among other methods, filing for patent applications on inventions that are important to the development and conduct of our business with the U.S. Patent and Trademark Office, or USPTO, and its foreign counterparts.

With respect to A-101, we own two issued U.S. patents, one issued patent in each of Australia, Germany, United Kingdom, India, New Zealand, Mexico, and Singapore, and a pending U.S. and PCT patent application. We do not currently rely on licenses to any third party's intellectual property for A-101. The two U.S. patents include claims that cover the use of high-concentration hydrogen peroxide for the alleviation of SK and acrochordons. The patents in Australia, New Zealand and India include claims that cover the use of high-concentration hydrogen peroxide for the alleviation of various skin conditions, including SK, acrochordons, corns, tags, acne, warts and rosacea. The patents in Germany, the United Kingdom, Mexico and Singapore include claims that cover the use of high-concentration hydrogen peroxide for the alleviation of acrochordons. The issued patents relating to the use of A-101 begin to expire in 2022, subject to any applicable patent term extension that may be available in a particular country.

Our pending U.S. and PCT patent application are directed to various formulations comprising high-concentration hydrogen peroxide, dosing regimens for such formulations, applicators for use with such formulations, and methods of treating various skin conditions, including SK and common warts, by the topical administration of such formulations. We plan to pursue the PCT application in numerous foreign countries, including in the European Union. Any claims that issue from these formal filings will expire in 2035, subject to any applicable patent term adjustment or extension that may be available in a particular country.

With respect to the JAK inhibitors we licensed from Rigel, we exclusively licensed in the field of dermatology multiple families of patents and applications relating to these compounds and the uses thereof. In particular, we exclusively licensed patents and applications with claims that specifically cover the composition of matter for these compounds in the United States, the European Union, and other major foreign markets. The issued patents specifically directed to these compounds begin to expire in 2030, subject to any applicable patent term adjustment or extension that may be available in a particular country. We also exclusively licensed applications in the United States, Australia,

Canada, Europe and Japan with claims that cover the use of these compounds for the treatment of autoimmune alopecia. Any claims that issue from these applications will expire in 2034, subject to any applicable patent term adjustment or extension that may be available in a particular country. We also licensed a family of patents and applications that relate to A-201 and A-301 that expire in 2023, subject to any applicable patent term adjustment or extension that may be available in a particular country.

With respect to the JAK inhibitors that we have licensed from other third parties, we exclusively licensed in the field of dermatology a family of applications relating to specific JAK 3 inhibitors and methods of use. In particular, we exclusively licensed a U.S. patent with claims directed to the use of these specific JAK 3 inhibitors to inhibit activity of JAK 3 in a patient, which patent expires in 2030, subject to any applicable patent term extension that may be available. We also exclusively licensed patent applications in the United States, Canada and Europe relating to these specific JAK 3 inhibitors and methods of use, which, if they were to issue as patents, would expire in 2030, subject to any applicable patent term adjustment or extension that may be available in a particular country.

We also use other forms of protection, such as trademark, copyright, and trade secret protection, to protect our intellectual property, particularly where we do not believe patent protection is appropriate or obtainable. We aim to take advantage of all of the intellectual property rights that are available to us and believe that this comprehensive approach will provide us with proprietary positions for our drug candidates, where available.

Table of Contents

Patents extend for varying periods according to the date of patent filing or grant and the legal term of patents in various countries where patent protection is obtained. The actual protection afforded by a patent, which can vary from country to country, depends on the type of patent, the scope of its coverage and the availability of legal remedies in the country. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, a patent term may be shortened if a patent is terminally disclaimed over another patent or as a result of delays in patent prosecution by the patentee, and a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in granting a patent or by patent term extension, which compensates a patentee for delays at the FDA. The patent term of a European patent is 20 years from its filing date; however, unlike in the United States, the European patent does not grant patent term adjustments. The European Union does have a compensation program similar to patent term extension called supplementary patent certificate that would effectively extend patent protection for up to five years.

We also protect our proprietary information by requiring our employees, consultants, contractors and other advisors to execute nondisclosure and assignment of invention agreements upon commencement of their respective employment or engagement. Agreements with our employees also prevent them from bringing the proprietary rights of third parties to us. In addition, we also require confidentiality or service agreements from third parties that receive our confidential information or materials.

Assignment Agreement and Finder's Services Agreement

In August 2012, we entered into an assignment agreement with the Estate of Mickey Miller, or the Miller Estate, under which we acquired some of the intellectual property rights covering A-101. The assignment of intellectual property rights covers specified know-how, along with modifications of, improvements to and variations on A-101 that meet defined chemical properties. Under the agreement, we have the sole and exclusive right, but not the duty, to develop, obtain regulatory approval for and commercialize A-101 in various countries throughout the world. We are required to use commercially reasonable efforts to develop and commercialize at least one product for at least one indication in the United States. In connection with obtaining the assignment of the intellectual property from the Miller Estate, we also entered into a separate finder's services agreement with KPT Consulting, LLC.

Under the terms of the assignment agreement and the finder's services agreement, we made aggregate upfront payments of $0.6 million in 2012 and one-time milestone payments of $0.4 million in 2013 upon the dosing of the first human subject with A-101 in our Phase 2 clinical trial. There are no remaining potential milestone payments under the assignment agreement. Under the finder's services agreement, we made a one-time milestone payment of $0.3 million in February 2016 upon the dosing of the first human subject with A-101 in our Phase 3 clinical trial, and we are obligated to make additional milestone payments of up to $1.0 million in the aggregate upon the achievement of specified development and regulatory milestones and up to $4.5 million upon the achievement of specified commercial milestones. Under each of the assignment agreement and the finder's services agreement, we are also obligated to pay royalties on sales of A-101 or related products, at low single-digit percentages of net sales, subject to reduction in specified circumstances. We have not made any royalty payments to date under either agreement. Both

agreements will terminate upon the expiration of the last pending, viable patent claim of the patents acquired under the assignment agreement, but no sooner than 15 years from the effective date of the agreements.

License Agreement with Rigel

In August 2015, we entered into an exclusive, worldwide license and collaboration agreement with Rigel for the development and commercialization of products containing two specified JAK inhibitors. Under this agreement, we intend to develop these JAK inhibitors for the treatment of AA and potentially for other dermatological conditions. We paid Rigel an upfront non-refundable payment of $8.0 million and 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, 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.

Table of Contents

The agreement terminates on the date of expiration of all royalty obligations unless earlier terminated by either party for a material breach. We may also terminate the agreement without cause at any time upon advance written notice to Rigel. Rigel, after consultation with us, will be responsible for maintaining and prosecuting the patent rights, and we 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 we jointly develop intellectual property, we 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.

Government Regulation and Product Approval

Governmental authorities in the United States, at the federal, state and local level, and analogous authorities in other countries extensively regulate, among other things, the research, development, testing, manufacture, safety surveillance, efficacy, quality control, labeling, packaging, distribution, record keeping, promotion, storage, advertising, distribution, marketing, sale, export and import, and the reporting of safety and other post-market information of products such as the one we are developing. A drug candidate, such as A-101, must be approved by the FDA before it may be legally promoted in the United States and by comparable foreign regulatory authorities before marketing in other jurisdictions. A-101 and any future drug candidates we may develop will be subject to similar requirements in other countries outside of the European Union and the United States prior to marketing in those countries. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations require the expenditure of substantial time and resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by regulatory authorities to approve applications, withdrawal of an approval, imposition of a clinical hold, import/export delays, issuance of warning letters and untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by FDA and the Department of Justice or other governmental entities.

United States Government Regulation

NDA Approval Processes

In the United States, the FDA regulates drug and medical device products under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. Our drug candidates are comprised of both a drug component (the hydrogen peroxide solution or gel) and a pen-type applicator. In the case of our drug candidates, the FDA's Center for Drug Evaluation and Research has primary jurisdiction over the premarket development, review and approval of our drug candidates. Accordingly, we are investigating our drug candidates pursuant to IND applications and expect to seek approval through the NDA pathway. Based on our discussions with the FDA to date, we do not

anticipate that the FDA will require us to submit a separate marketing application for the pen-type applicator that will be used with our drug candidates, but this could change during the course of the FDA's review of our NDA.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice regulations;
- submission to the FDA of an IND which must take effect before clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before clinical testing may be initiated at the clinical site;
- performance of adequate and well-controlled clinical trials in accordance with good clinical practice, or GCP, regulations to establish the safety and efficacy of the proposed drug product for each indication;
- preparation and submission to the FDA of an NDA;
- review of the NDA by a FDA advisory committee, if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product or its components are produced to assess compliance with current good manufacturing practices, or

Table of Contents

cGMP, regulations to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;

- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including potential requirements for a risk evaluation and mitigation strategy and post-approval studies required by the FDA.

Once a drug candidate is identified for development, it enters the preclinical or nonclinical testing stage. Preclinical studies include laboratory evaluations of product chemistry, pharmacology, toxicity and formulation. An IND sponsor must submit the results of the preclinical studies, together with manufacturing information and analytical data, to the FDA as part of the IND. Some preclinical studies may continue even after the IND is submitted. In addition to including the results of the preclinical studies, the IND will also include a protocol detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated if the first phase lends itself to an efficacy determination. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, places the IND on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin. A clinical hold may occur at any time during the life of an IND, and may affect one or more specific clinical trials or all clinical trials conducted under the IND.

All clinical trials must be conducted under the supervision of one or more qualified investigators in accordance with current Good Clinical Practices regulations. They must be conducted under protocols detailing the objectives of the trial, dosing procedures, research subject selection and exclusion criteria and the safety and effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND, and progress reports detailing the status of the clinical trials must be submitted to the FDA annually. Sponsors also must timely report to FDA serious and unexpected adverse reactions, any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure, or any findings from other studies or animal or in vitro testing that suggest a significant risk in humans exposed to the drug. An institutional review board, or IRB, at each institution participating in the clinical trial must review and approve the protocol before the clinical trial commences at that institution and must also approve the information regarding the trial and the consent form that must be provided to each research subject or the subject's legal representative, monitor the study until completed and otherwise comply with IRB regulations.

Clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1. The drug is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and elimination. In the case of some products for severe or life-threatening diseases, such as cancer, and especially when the product may be inherently too toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients who already have the condition.
- Phase 2. Clinical trials are performed on a limited patient population intended to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3. If a drug candidate is found to be potentially effective and to have an acceptable safety profile in Phase 2 clinical trials, the clinical trial program will be expanded to Phase 3 clinical trials to further evaluate dosage, clinical

efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These studies are intended to establish the overall risk-benefit ratio of the product and provide an adequate basis for product approval and labeling claims.

Phase 4 clinical trials are conducted after approval to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of drugs approved under accelerated approval regulations, or when otherwise requested by the FDA in the form of post-market requirements or commitments. Failure to promptly conduct any required Phase 4 clinical trials could result in withdrawal of approval.

Clinical trials are inherently uncertain and Phase 1, Phase 2 and Phase 3 testing may not be successfully completed. The FDA or the sponsor may suspend a clinical trial at any time for a variety of reasons, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. In some cases, clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, which is called the clinical monitoring board or data safety monitoring board. This group provides authorization for whether or not a trial

Table of Contents

may move forward at designated check points. These decisions are based on the limited access to data from the ongoing trial.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to the submission of an IND, at the end-of-Phase 2 and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date and for the FDA to provide advice on the next phase of development. Sponsors typically use the meeting at the end-of-Phase 2 to discuss their Phase 2 clinical trial results and present their plans for the pivotal Phase 3 clinical trial or trials that they believe will support the approval of the new drug.

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

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests and other control mechanisms, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of user fees, but a waiver of such fees may be obtained under specified circumstances. The FDA reviews all NDAs submitted for a period of 60 days to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing.

During the approval process, the FDA also will determine whether a risk evaluation and mitigation strategy, or REMS, is necessary to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the application must submit a proposed REMS, and the FDA will not approve the application without an approved REMS, if required. A REMS can substantially increase the costs of obtaining approval. The FDA could also require a special warning, known as a boxed warning, to be included in the product label in order to highlight a particular safety risk.

Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant. The FDA may refer the NDA to an advisory committee for review and recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. NDAs receive either standard or priority review. A drug representing a significant improvement in treatment, prevention or diagnosis

of disease may receive priority review. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on the NDA from ten months to six months from FDA filing of the NDA. After the FDA evaluates the NDA and conducts inspections of manufacturing facilities where the drug product and/or its API will be produced, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. Even if such data and information are submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval.

Post-approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA and other governmental agencies, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements is not maintained or if problems occur after the product reaches the market. Later discovery of

Table of Contents

previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further FDA review and approval. There also are continuing, annual user fee requirements for products and the establishments at which such products are manufactured, as well as new application fees for certain supplemental applications. In addition, the FDA may require testing and surveillance programs to monitor the effect of approved products that have been commercialized, and the FDA has the power to prevent or limit further marketing of a product based on the results of these post-marketing programs.

Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and some state agencies for compliance with GMP regulations and other laws. The FDA has promulgated specific requirements for drug cGMPs and device cGMPs embodied in the Quality System Regulation. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Failure to comply with the applicable United States requirements at any time during the product development process or approval process, or after approval, may subject us to administrative or judicial sanctions, any of which could have a material adverse effect on us. These sanctions could include:

- refusal to approve pending applications;
- withdrawal of an approval;
- imposition of a clinical hold;
- warning letters;
- product seizures or detention, or refusal to permit the import or export of products;
- restrictions on the marketing or manufacturing of the product;
- total or partial suspension of production or distribution or product recalls; or
- injunctions, fines, disgorgement, or civil or criminal penalties.

The FDA strictly regulates the marketing, labeling, advertising and promotion of drug products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. In

addition, FDA regulations and guidance are often issued revised or reinterpreted by the agency in ways that may significantly affect our business and our products. It is impossible to predict whether legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be issued or changed or what the impact of such changes, if any, may be.

Non-patent Exclusivity

The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity, or NCE. A drug is an NCE if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. Because we believe that an NDA has never been approved for hydrogen peroxide, we believe that our product qualifies as an NCE and is entitled to a five-year period of market exclusivity under the FDCA if approved, but FDA may disagree with our interpretation.

If market exclusivity is granted, during the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-

Table of Contents

infringement to one of the patents listed with the FDA by the innovator NDA holder. The FDCA also provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages, dosage forms or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of an NDA. However, an applicant submitting an NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to regulations of other countries governing our business activities, including, our clinical trials and the commercial sale and distribution of our product. Even if we obtain FDA approval for a product, we must obtain approval by the comparable regulatory authorities of countries outside of the United States before we can commence clinical trials in such countries and approval of the regulators of such countries or economic areas, such as the European Union before we may market products in those countries or areas. The approval process and requirements governing the conduct of clinical trials, product licensing and promotion, pricing and reimbursement vary greatly by geographic region, and the time may be longer or shorter than that required for FDA approval.

In the European Economic Area, or EEA, which is composed of the 28 Member States of the European Union plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA.

There are two types of MAs:

· The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency, or EMA, and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, and medicinal products indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU. Under the Centralized Procedure, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when the authorization of a medicinal product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation.

Under the accelerated procedure, the standard 210 days review period is reduced to 150 days.

- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure.

In the EEA, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity.

Table of Contents

Other Healthcare Laws

Although we currently do not have any products on the market we are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, physician sunshine and privacy and security laws.

The federal Anti-Kickback Statute makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term "remuneration" has been broadly interpreted to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, formulary managers, and beneficiaries on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the Anti-Kickback Statute has been violated. Violations of this law are punishable by up to five years in prison, and can also result in criminal fines, civil money penalties, administrative penalties and exclusion from participation in federal healthcare programs.

Additionally, the intent standard under the Anti-Kickback Statute was amended by the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, collectively the Affordable Care Act, to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the Affordable Care Act codified case law 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 federal civil False Claims Act.

Federal false claims and false statement laws, including the federal civil False Claims Act, prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent or not provided as claimed. Entities can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers, promoting a product off-label, or for providing medically unnecessary services or items. In addition, our future activities relating to the sale and marketing of our product are subject to scrutiny under this law. Penalties for the federal civil False Claims Act violations may include up to three times the actual damages sustained by the government, plus mandatory civil penalties of between $5,500 and $11,000 for each separate false claim, the potential

for exclusion from participation in federal healthcare programs, and, although the federal False Claims Act is a civil statute, False Claims Act violations may also implicate various federal criminal statutes. The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the Anti-Kickback Statute, the Affordable Care Act amended the intent standard for certain healthcare fraud statutes under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Table of Contents

Also, many states have similar fraud and abuse statutes or regulations that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

There has also been a recent trend of increased federal and state regulation of payments made to physicians and other healthcare providers. The Affordable Care Act imposed, among other things, new annual reporting requirements for covered manufacturers for certain payments and other transfers of value provided to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Failure to submit timely, accurately and completely the required information for all payments, transfers of value and ownership or investment interests may result in civil monetary penalties of up to an aggregate of $150,000 per year and up to an aggregate of $1 million per year for "knowing failures." Covered manufacturers must submit reports to the Centers for Medicare and Medicaid Services by the 90th day of each calendar year. Certain states also mandate implementation of compliance programs, impose restrictions on drug manufacturer marketing practices and/or require the tracking and reporting of gifts, compensation and other remuneration to physicians.

Because we intend to commercialize a product that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we intend to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject. Although the development and implementation of compliance programs designed to establish internal controls and facilitate compliance can mitigate the risk of investigation, prosecution, and penalties assessed for violations of these laws, or any other laws that may apply to us, the risks cannot be entirely eliminated. If our operations are found to be in violation of any of such laws or any other governmental regulations, we may be subject to penalties, including, without limitation, administrative, civil, and criminal penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and individual imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

We may also be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, including the final omnibus rule published on January 25, 2013, mandates, among other things, the adoption of uniform standards for the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information, which require the adoption of administrative, physical and technical safeguards to protect such information. Among other things, HITECH makes HIPAA's security standards directly applicable to "business associates", namely independent contractors or agents of covered entities that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities and business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties.

Health Care Reform

In the United States, there have been and continue to be a number of significant legislative initiatives to contain healthcare costs. For example, in March 2010, the Affordable Care Act was passed, which has had, and is expected to continue to have, a significant impact on the healthcare industry. The Affordable Care Act was designed to expand coverage for the uninsured while at the same time containing overall healthcare costs. With regard to pharmaceutical products, among other things, the Affordable Care Act expanded and increased industry rebates for drugs covered under Medicaid programs; addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; extended the rebate program to individuals enrolled in Medicaid managed care organizations; established annual fees and taxes on manufacturers of certain branded prescription drugs; and made changes to the coverage requirements under the Medicare prescription drug benefit; and established 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

Table of Contents

beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D. Moreover, the Affordable Care Act provided incentives to programs that increase the federal government's comparative effectiveness research and implemented payment system reforms including a national pilot program on payment bundling to encourage hospitals, physicians and other providers to improve the coordination, quality and efficiency of certain healthcare services through bundled payment models. Since its enactment there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to it in the future. We continue to evaluate the effect that the Affordable Care Act will have on our business. In the coming years, additional legislative and regulatory changes could be made to governmental health programs that could significantly impact pharmaceutical companies and the success of our drug candidates.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least $1.2 trillion for fiscal years 2012 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, cancer treatment centers and imaging centers. Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. More recently, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products. For example, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug product.

The Affordable Care Act, as well as other federal and state healthcare reform measures that have been and may be adopted in the future, could harm our future revenue. Additional legislative actions may be taken in the future which may change current regulations, guidance and interpretations. The impact of such actions on our business, if any, cannot presently be determined.

The Hatch Waxman Amendments to the FDC Act

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product or a method of using the product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an ANDA or an application covered by Section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act, or FDCA. An ANDA provides for marketing of a drug product that has the same active ingredients, generally in the same strengths and dosage form, as the listed drug and has been shown through pharmacokinetic, or PK, testing to be bioequivalent to the listed drug. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are generally not required to conduct, or submit results of, preclinical studies or clinical tests to prove the safety or effectiveness of their drug product. Section 505(b)(2) applications provide for marketing of a drug product that may have the same active ingredients as the listed drug and contains full safety and effectiveness data as an NDA, but at least some of this information comes from studies not conducted by or for the applicant. This alternate regulatory pathway enables the applicant to rely, in part, on the FDA's findings of safety and efficacy for an existing product, or published literature, in support of its application. The FDA may then approve the new drug candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication sought by the 505(b)(2) applicant.

Table of Contents

The ANDA or Section 505(b)(2) applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA or Section 505(b)(2) applicant may also elect to submit a statement certifying that its proposed ANDA label does not contain, or carves out, any language regarding a patented method of use rather than certify to such listed method of use patent. If the applicant does not challenge the listed patents by filing a certification that the listed patent is invalid or will not be infringed by the new product, the ANDA or Section 505(b)(2) application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA or Section 505(b)(2) applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA or Section 505(b)(2) application has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA or Section 505(b)(2) application until the earliest of 30 months, expiration of the patent, settlement of the lawsuit, and a decision in the infringement case that is favorable to the ANDA or Section 505(b)(2) applicant. This prohibition is generally referred to as the 30-month stay. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes and the reference drug sponsor's decision to initiate patent litigation.

The ANDA or Section 505(b)(2) application also will not be approved until any applicable non patent exclusivity listed in the Orange Book for the referenced product has expired.

We intend to list any patents that are eligible for listing in the Orange Book in our NDA.

Patent Term Extension

In the United States, after NDA approval, owners of relevant drug patents may apply for up to a five year patent extension, which provides patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent. The allowable patent term extension is calculated as half of the drug's testing phase, which is the time between the IND submission becoming effective and the NDA submission, and all of the review phase, which is the time between NDA submission and approval, up to a maximum extension of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended.

Similar provisions are available in the European Union and other foreign jurisdictions to extend the term of a patent that covers an approved drug. For example, in Japan, it may be possible to extend the patent term for up to five years and in the European Union, it may be possible to obtain a supplementary patent certificate that would effectively extend patent protection for up to five years. In the future, if our drug candidates receive FDA approval, we expect to apply for patent term extensions on patents covering those drugs.

Coverage and Reimbursement

We do not expect third-party payors to cover and reimburse customers 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 with 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. By contrast, 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

Table of Contents

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 product.

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.

In the United States, no uniform policy of coverage and reimbursement for medical procedures exists among third-party payors. Therefore, coverage and reimbursement for procedures can differ significantly from payor to payor. Decisions regarding the extent of coverage and amount of reimbursement to be provided for an in-office procedure to remove warts are made on a plan by plan basis. One payor's determination to provide coverage for a procedure does not assure that other payors will also provide coverage, and adequate reimbursement.

In addition to uncertainties surrounding coverage policies, there are periodic changes to reimbursement. Third-party payors regularly update reimbursement amounts and also from time to time revise the methodologies used to determine reimbursement amounts. This includes annual updates to payments to physicians for procedures during which our drug candidates will be used. To the extent the procedure using our drug candidates would be covered, the cost of our drugs generally is 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. An example of payment updates is the Medicare program's updates to hospital and physician payments, which are 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. The Medicare Access and CHIP Reauthorization Act of 2015, or MACRA, ended the use of the statutory formula, and provided for a 0.5% annual increase in payment rates under the Medicare Physician Fee Schedule through 2019, but no annual update from 2020 through 2025. MACRA also introduced a merit based incentive bonus program for Medicare physicians beginning in 2019. At this time it is unclear how the introduction of the merit based incentive program will impact overall physician reimbursement under the Medicare program.

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.

Employees

As of December 31, 2015, we had 18 employees. All of our employees are located in the United States. None of our employees is represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Information about Segments

We currently operate in one business segment. Our singular focus is identifying, developing and commercializing innovative and differentiated drugs to address significant unmet needs in dermatology. See “Note 2—Summary of Significant Accounting Policies—Segment Data” to our consolidated financial statements contained in Part II, Item 8 of this Annual Report.

Table of Contents

Corporate Information

We were incorporated under the laws of the State of Delaware in July 2012. Our principal executive offices are located at 101 Lindenwood Drive, Suite 400, Malvern, PA 19355. Our telephone number is (484) 324-7933. We completed our initial public offering in October 2015 and our common stock is listed on the NASDAQ Global Select Market under the symbol “ACRS”.

Available Information

Our internet website address is www.aclaristx.com. In addition to the information contained in this Annual Report, information about us can be found on our website. Our website and information included in or linked to our website are not part of this Annual Report.

Our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge through our website as soon as reasonably practicable after they are electronically filed with or furnished to the Securities and Exchange Commission, or SEC. The public may read and copy the materials we file with the SEC at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. Additionally the SEC maintains an internet site that contains reports, proxy and information statements and other information. The address of the SEC’s website is www.sec.gov.

Item 1A. Risk Factors

Our business is subject to numerous risks. You should carefully consider the following risks and all other information contained in this Annual Report, as well as general economic and business risks, together with any other documents we file with the SEC. If any of the following events actually occur or risks actually materialize, it 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 $20.6 million and $8.5 million for the years ended December 31, 2015 and 2014, respectively. As of December 31, 2015, we had an accumulated deficit of $42.8 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, and we have initiated two multi-center, double-blind Phase 3 clinical trials and one open-label clinical trial 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, and in the fourth quarter of 2015 we initiated a Phase 2 clinical trial to evaluate A-101, for the treatment of common warts. Additionally, we are developing 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 and A-101 for the treatment of common warts;

Table of Contents

- 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-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 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 conduct 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.

Table of Contents

As of December 31, 2015, we had cash, cash equivalents and marketable securities of $92.0 million. We believe that our existing cash and cash equivalents as of the date of this Annual Report will enable us to fund our operating expenses and capital expenditure requirements for a period greater than 12 months from December 31, 2015 based on our current operating assumptions. These assumptions 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;
- the progress and results of the 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
- 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 commercially acceptable 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, developing A-101 for the treatment of SK, including undertaking preclinical studies and conducting clinical trials, and acquiring new drug candidates and related intellectual property. A-101 for the treatment of SK is our only drug candidate

Table of Contents

for which we have completed 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 completed 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 completed 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:

- 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

Table of Contents

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. In the first quarter of 2016, we initiated two multi-center, double-blind Phase 3 clinical trials of A-101 in patients with SK lesions on the face, trunk and extremities and a third open-label Phase 3 clinical trial. The development of our other drug candidates is less advanced and we have not completed 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.

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.

Table of Contents

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.

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

Table of Contents

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.

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

Table of Contents

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.

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.

Table of Contents

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. We are also aware of early research being conducted with Akt inhibitors as a potential treatment for 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.

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 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.

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.

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

Table of Contents

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. The Medicare Access and CHIP Reauthorization Act of 2015, or MACRA, ended the use of the statutory formula, and provided for a 0.5% annual increase in payment rates under the Medicare Physician Fee Schedule through 2019, but no annual update from 2020 through 2025. MACRA also introduced a merit based incentive bonus program for Medicare physicians beginning in 2019. At this time it is unclear how the introduction of the merit based incentive program will impact overall physician reimbursement under the Medicare program. Any resulting decrease in payment under the merit based reimbursement system may adversely affect our revenue and results of operations. In addition, the Medicare Physician 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.

Table of Contents

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 Phase 3 clinical trials of A-101 and expect to engage a CRO to conduct clinical trials 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

Table of Contents

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. 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

Table of Contents

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 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

Table of Contents

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.

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

Table of Contents

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 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 agreements, 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. We have also entered into a license agreement with other third parties for patent rights and other intellectual property relating to

Table of Contents

JAK inhibitors. If we materially breach or fail to perform any provision under these license agreements, including failure to make payments when due for royalties and failure to use commercially reasonable efforts to develop and commercialize a JAK inhibitor, the counterparty has the right to terminate our license, and upon the effective date of such termination, our right to practice the licensed patent rights and other intellectual property would end. Any uncured, material breach under the license agreements could result in our loss of rights to practice the patent rights and other intellectual property licensed to us, 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, including those being used in the development of A-201 and A-301, are currently covered in patents and applications in the United States, the European Union, and other major foreign markets. 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.

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 licensed intellectual property from Rigel in the field of dermatology related to our JAK inhibitors, A-201 and A-301. We also exclusively licensed intellectual property from other third parties related to other novel 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.

Third parties may develop JAK inhibitors, including those related to our drug candidates, outside of the field of dermatology.

We exclusively license intellectual property from Rigel and other third parties in order to develop, use, manufacture, sell and commercialize our JAK inhibitors in the field of dermatology. These third parties have retained the rights under such intellectual property to develop, use, manufacture, sell and commercialize the licensed JAK inhibitors outside of the field of dermatology. If any of these third parties were to 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. Our licensors also retained the intellectual

Table of Contents

property rights to develop, use, manufacture, sell and commercialize other structurally similar JAK inhibitors. If such third parties 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.

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

Table of Contents

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. 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

Table of Contents

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. The issued U.S. patent relating to the use of our novel JAK inhibitors licensed from other third parties is scheduled to expire in 2030. Given the 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.

Table of Contents

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 intend to commercialize 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;
- 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.

Table of Contents

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.

Table of Contents

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;
- 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;

Table of Contents

- 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, 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

Table of Contents

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;
- 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.

Since its enactment there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to it in the future, and we will continue to assess the Affordable Care Act's impact on us as additional regulations, guidance, and orders are issued.

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 2025 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.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. In addition, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug product. 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

Table of Contents

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. 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.

Table of Contents

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 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.

Table of Contents

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.

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 December 31, 2015, we had 18 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

Table of Contents

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 Phase 3 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;
- 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,

Table of Contents

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.

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.

Table of Contents

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.

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

Table of Contents

- 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 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, 2015, we had federal and state net operating loss carryforwards of $13.9 million for each, and federal research and development tax credit carryforwards of $0.5 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

Table of Contents

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.

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

Table of Contents

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.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

We currently sublease 9,436 square feet of space for our headquarters in Malvern, Pennsylvania, which will increase to 11,659 square feet effective June 1, 2016. Our sublease has a term through November 30, 2019, subject to renewal for at least two six-month terms. We sublease this space from an entity affiliated with some of our executive officers and directors. We believe that our current facilities are suitable and adequate to meet our current needs. We intend to add new facilities or expand existing facilities as we add employees, and we believe that suitable additional or substitute space will be available as needed to accommodate any such expansion of our operations.

Item 3. Legal Proceedings

We are not subject to any material legal proceedings.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

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

Market Information for Common Stock

Our common stock commenced trading on The NASDAQ Global Select Market under the symbol "ACRS" on October 7, 2015. Prior to our initial public offering, there was no public market for our common stock. The following table sets forth for the periods indicated the high and low sales prices of our common stock as reported on The NASDAQ Global Select Market.

	High	Low
2015		
Fourth Quarter (since October 7, 2015)	$ 33.88	$ 10.99

On March 22, 2016, the last reported bid price for our common stock was $15.37 per share.

Dividend Policy

We have never declared or paid any dividends on our common stock. We anticipate that we will retain all of our future earnings, if any, for use in the operation and expansion of our business and do not anticipate paying cash dividends in the foreseeable future.

Stockholders

As of March 22, 2016, we had 20,157,503 shares of common stock outstanding held by 58 holders of record.

Performance Graph

The following graph compares the performance of our common stock since October 7, 2015, the date of which our common stock commenced trading on The NASDAQ Global Select Market, with the performance of the NASDAQ

66

Table of Contents

Composite Index (U.S.) and the NASDAQ Biotechnology Index. The comparison assumes a $100 investment on October 7, 2015 in our common stock, the stocks comprising the NASDAQ Composite Index, and the stocks comprising the NASDAQ Biotechnology Index, and assumes reinvestment of the full amount of all dividends, if any. Historical stockholder return is not necessarily indicative of the performance to be expected for any future periods.

Comparison of Cumulative Total Return

Among Aclaris Therapeutics, Inc., the NASDAQ Composite Index and the NASDAQ Biotechnology Index

The performance graph shall not be deemed to be incorporated by reference by means of any general statement incorporating by reference this Form 10-K into any filing under the Securities Act of 1933, as amended or the Exchange Act, except to the extent that we specifically incorporate such information by reference, and shall not otherwise be deemed filed under such acts.

Use of Proceeds from Initial 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 $2.3 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. Through December 31, 2015, we have not used any of the net proceeds, as we have used cash on hand as of the initial public offering date to fund the continued research and development of A-101 and our other drug candidates and for working capital and other general corporate purposes.

Table of Contents

Recent Sales of Unregistered Securities

From January 1, 2015 through October 6, 2015, we granted options to purchase an aggregate of 640,262 shares of our common stock under our 2012 equity compensation plan with a weighted average 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.

Purchases of Equity Securities by the Issuer and Affiliated Parties

None.

Item 6. Selected Consolidated Financial Data

The following selected consolidated financial data as of and for the years ended December 31, 2015, 2014 and 2013 is derived from our audited consolidated financial statements included within this Annual Report. The balance sheet data as of December 31, 2013 has been derived from our audited consolidated financial statements which are not included herein. Our historical results are not necessarily indicative of the results to be expected in the future. The selected financial data should be read together with Item 7. “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and in conjunction with the consolidated financial statements, related notes, and other financial information included elsewhere in this Annual Report.

	Year Ended December 31,		
	2015	2014	2013
	(in thousands)		
Consolidated Statement of Operations Data:			
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	15,339	6,507	3,488
General and administrative	5,328	2,026	1,769
Total operating expenses	20,667	8,533	5,257
Loss from operations	(20,667)	(8,533)	(5,257)
Interest income	104	16	21
Loss on foreign currency exchange	—	—	—
Net loss	(20,563)	(8,517)	(5,236)
Accretion of convertible preferred stock	(2,566)	(2,054)	(1,740)
Net loss attributable to common stockholders	$ (23,129)	$ (10,571)	$ (6,976)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.79)	$ (6.15)	$ (6.45)
Weighted average common shares outstanding, basic and diluted	6,107,042	1,720,082	1,081,347

Table of Contents

	As of December 31,		
	2015	2014	2013
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 92,038	$ 16,648	$ 14,126
Working capital (a)	84,969	14,883	13,019
Total assets	94,076	17,377	14,207
Convertible preferred stock	—	36,677	23,000
Total stockholders' equity (deficit)	92,521	(20,755)	(9,163)

(a) Working capital is defined as current assets minus current liabilities

On September 24, 2015, we effected a 1 for 3.45 reverse stock split of our issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of our then-outstanding convertible preferred stock. Accordingly, all share and per share amounts for all periods presented have been adjusted retroactively, to reflect this reverse stock split.

On October 13, 2015, we sold 5,750,000 shares of common stock in our initial public offering.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the consolidated financial statements and the related notes to those statements included later in this Annual Report. In addition to historical financial information, the following discussion contains forward looking statements that reflect our plans, estimates, beliefs and expectations that involve risks and uncertainties. Our actual results and the timing of events could differ materially from those discussed in these forward looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this Annual Report, particularly in "Item 1A. Risk Factors" and "Special Note Regarding Forward Looking Statements."

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 Topical Solution, 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 statistically significant improvements in clearing SK lesions on the face, trunk and extremities of the body. In the first quarter of 2016, we initiated two multi-center, double-blind Phase 3 clinical trials and one open label Phase 3 clinical trial of A-101 in patients with SK. If the results of these trials are favorable, we plan 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, also known as verruca vulgaris, and A-102, a proprietary gel dosage form of hydrogen peroxide, as a prescription treatment for SK and common warts. In the fourth quarter of 2015, we initiated a Phase 2 clinical trial to evaluate A-101 for the treatment of common warts. We have also 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 and technologies 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 current or future drug candidates.

Table of Contents

Since our inception, we have incurred significant operating losses. Our net losses were $20.6 million, $8.5 million and $5.2 million for the years ended December 31, 2015, 2014 and 2013, respectively. As of December 31, 2015, we had an accumulated deficit of $42.8 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 have incurred and expect to continue 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 commercially acceptable 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 for the development and commercialization of products containing two specified JAK inhibitors. Under this agreement, we intend to develop these JAK inhibitors for the treatment of alopecia areata, or AA, and potentially for other dermatological conditions. We paid Rigel an upfront nonrefundable payment of $8.0 million in September 2015. 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 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, 10 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. We may also terminate the agreement without cause at any time upon advance written notice to Rigel. Rigel, after consultation with us, will be responsible for maintaining and prosecuting the patent rights, and we 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 we jointly develop intellectual property, we 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.

We accounted for the license and collaboration agreement with Rigel as an asset acquisition since the 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 year ended December 31, 2015. We will record contingent milestone payments and royalties as research and development expense or cost of revenues, respectively, in the period in which such liabilities are incurred.

We concluded the 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, we did not acquire employees, tangible assets, 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 expensed the acquired intellectual property asset upon acquisition because we will use it in our research and development activities and believe it has no alternative future uses.

Table of Contents

Other 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 the assignment agreement for the acquisition of intellectual property related to A-101, we also entered into a finder's services agreement under which we have paid aggregate milestone payments of $0.5 million and have agreed to make aggregate payments of up to $1.0 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.

In November 2015, we entered into an exclusive, worldwide license agreement with third parties for the development and commercialization of products containing specified novel JAK inhibitors for any therapeutic, prophylactic, prognostic or diagnostic applications for use in any dermatological condition or disease. We made upfront non-refundable payments of $0.25 million and are obligated to make aggregate payments of up to $2.35 million upon the achievement of specified milestones, such as completion of clinical trials and filing of applications for regulatory approvals. With respect to any products we commercialize under the agreement, we will pay tiered royalties equal to a low to middle single-digit percentage of annual net sales, subject to specified reductions, as determined on a country-by-country and product-by-product basis, until the date that all of the patent rights for that product have expired, or in specified countries under specified circumstances, ten years from the first commercial sale of such product.

Components of Our Results of Operations

Revenue

We have not generated any revenue since our inception and do not expect to generate 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;
- outsourced professional scientific development services;
- employee-related expenses, which include salaries, benefits and stock-based compensation;
- depreciation of manufacturing equipment;
- payments made under our agreements with third parties under which we have acquired or licensed intellectual property;
- expenses relating to regulatory activities, including filing fees paid to regulatory agencies; and
- laboratory materials and supplies used to support our research activities

Table of Contents

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. We expect our research and development expenses to increase significantly over the next several years as we increase personnel costs, including stock-based compensation, continue Phase 3 clinical trials of A-101 in patients with SK, and conduct other clinical trials and prepare regulatory filings for our drug candidates.

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 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 and legal 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 Securities and Exchange Commission, or SEC, requirements, accounting and investor relations costs, 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 that drug candidate.

Interest Income

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

Table of Contents

Critical Accounting Policies and Significant Judgments and Estimates

Our financial statements are prepared in accordance with generally accepted accounting principles in the United States of America. The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of expenses during the reported period. 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. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers require advance payments; however, some invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in the financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and makes adjustments if necessary.

We base expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accounting for service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or prepaid accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to

our estimates of research and development expenses.

Stock-Based Compensation

We measure the compensation expense of stock-based awards granted to employees and directors using the grant date fair value of the award. We recognize compensation expense for share-based awards expected to vest on a straight-line basis over their requisite service period, which is generally the vesting period of the award. We have issued stock option and restricted stock unit awards with service-based vesting conditions.

We initially measure the compensation expense of stock-based awards granted to consultants using the grant date fair value of the award. We recognize compensation expense over the period during which services are rendered by the consultant. At the end of each financial reporting period prior to the completion of services being rendered, we re-measure the compensation expense related to these awards using the then current fair value of the stock-based award, based on updated assumptions in the Black-Scholes option-pricing model.

We measure the compensation expense of restricted stock unit awards using the closing market price for our common stock on the grant date of the award applied to the total number of units that are expected to vest.

Table of Contents

We estimate the fair value of each stock option grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of our stock options, the risk free interest rate for a period that approximates the expected term of our stock options and our expected dividend yield. We based expected volatility on historical volatility of comparable guideline public companies because of our brief history as a public company. We use the 'simplified' expected term calculation method described in SEC Staff Accounting Bulletin No. 107, Share-Based Payment, and Staff Accounting Bulletin No. 110, Share-Based Payment, because we have limited experience as a public company. We base the risk-free interest rate on the U.S. Treasury yield in effect at the time of grant for an instrument with a maturity that is commensurate with the expected term of the stock options. We use a dividend yield of zero as we have never paid cash dividends on our common stock, and have no present intention to pay cash dividends. Prior to our IPO, we valued our common stock using a hybrid method which used market approaches to estimate our enterprise value. The hybrid method used was a probability-weighted expected return method which is a scenario-based methodology that estimated the fair value of our common stock based upon an analysis of future values for the company assuming various outcomes. The hybrid method used calculated equity values using an option pricing model in one or more of scenarios, and also considered the rights of each class of stock.

Income Taxes

Since our inception in 2012, we have not recorded U.S. federal or state income tax benefits for the net operating losses we have incurred in each year or for our earned research and development tax credits, due to our uncertainty of realizing a benefit from those items. As of December 31, 2015, we had federal and state net operating loss carryforwards of $13.9 million each, both of which begin to expire in 2032. As of December 31, 2015, we also had federal research and development tax credit carryforwards of $0.5 million, which begin to expire in 2032, and we had no state research and development tax credit carryforwards.

Results of Operations

Comparison of Years Ended December 31, 2015 and 2014

The following table summarizes our results of operations for the years ended December 31, 2015 and 2014:

	Year Ended December 31,		
	2015	2014	Change
	(in thousands)		
Revenue	$ —	$ —	$ —

Operating expenses:			
Research and development	15,339	6,507	8,832
General and administrative	5,328	2,026	3,302
Total operating expenses	20,667	8,533	12,134
Loss from operations	(20,667)	(8,533)	(12,134)
Interest income	104	16	88
Net loss	$ (20,563)	$ (8,517)	$ (12,046)

Research and Development Expenses

Research and development expenses were $15.3 million for the year ended December 31, 2015, compared to $6.5 million for the year ended December 31, 2014. The increase of $8.8 million was primarily attributable to an $8.0 million upfront payment to Rigel in connection with our license of rights to specified JAK inhibitors and related intellectual property. In addition, regulatory and clinical consulting expenses increased by $0.4 million in connection with our Phase 2 trials of A-101, and depreciation expense increased by $0.4 million resulting from an equipment impairment charge of $0.3 million recorded in the year ended December 31, 2015.

General and Administrative Expenses

General and administrative expenses were $5.3 million for the year ended December 31, 2015, compared to $2.0 million for the year ended December 31, 2014. The increase of $3.3 million was primarily attributable to increases of $0.8 million in payroll related costs mainly associated with increased headcount, $0.6 million in stock-based

Table of Contents

compensation, $0.5 million in market research expenses, $0.5 million in patent filing and prosecution costs related to the JAK inhibitor technology, $0.8 million in professional fees for accounting and auditing services, investor relations, insurance and board fees associated with becoming a public company.

Interest Income

Interest income increased during the year ended December 31, 2015 as a result of higher invested cash and marketable securities balances following our IPO.

Comparison of Years Ended December 31, 2014 and 2013

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

	Year Ended December 31,		
	2014	2013	Change
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	6,507	3,488	3,019
General and administrative	2,026	1,769	257
Total operating expenses	8,533	5,257	3,276
Loss from operations	(8,533)	(5,257)	(3,276)
Interest income	16	21	(5)
Net loss	$ (8,517)	$ (5,236)	$ (3,281)

Research and Development Expenses

Research and development expenses were $6.5 million for the year ended December 31, 2014, compared to $3.5 million for the year ended December 31, 2013. The increase of $3.0 million was primarily attributable to an increase of $3.1 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 year, consisting of increases of $1.9 million in clinical expenses, $1.1 million in manufacturing scale-up expenses and $0.1 million in development-related expenses. We also had an increase of $0.1 million in personnel-related expenses. These increases were partially offset by a decrease of $0.2 million in expenses due to a $0.2 million milestone payment made in 2013 under our assignment agreement,

compared to no milestone payments made in 2014.

General and Administrative Expenses

General and administrative expenses were $2.0 million for the year ended December 31, 2014, compared to $1.8 million for the year ended December 31, 2013. The increase of $0.2 million was primarily attributable to increases of $0.1 million in market research expenses and $0.1 million in related-party management 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 $71.5 million of gross proceeds from sales of our convertible preferred stock and net proceeds of $56.6 million from our IPO in October 2015.

As of December 31, 2015, we had cash, cash equivalents and marketable securities of $92.0 million. 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 sublease obligations and contingent obligations under intellectual property licensing arrangements, each of which is described below.

Table of Contents

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 $2.3 million in connection with the IPO. 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 and 6 to our consolidated financial statements in this Annual Report on Form 10-K for additional details.

Cash Flows

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

	Year Ended December 31,		
	2015	2014	2013
	(in thousands)		
Cash used in operating activities	$ (20,369)	$ (7,636)	$ (4,920)
Cash used in investing activities	(76,951)	(1,779)	(4,535)
Cash provided by financing activities	96,414	10,584	—
Net (decrease) increase in cash and cash equivalents	$ (906)	$ 1,169	$ (9,455)

Operating Activities

During the year ended December 31, 2015, our operating activities used $20.4 million of cash, primarily resulting from our net loss of $20.6 million and net cash used in our operating assets and liabilities of $1.1 million, partially offset by non-cash increases of $0.9 million in stock-based compensation expense and a $0.3 million write-down of equipment held for sale to net realizable value. Net cash used in changes in our operating assets and liabilities during the year ended December 31, 2015 consisted primarily of a $1.3 million increase in prepaid expenses and other current assets and a $0.4 million decrease in accounts payable, partially offset by a $0.6 million increase in accrued expenses. The increase in accrued expenses was primarily due to the timing of invoices for licensing fees and legal expenses relating to our wholly-owned subsidiary. The increase in prepaid expenses and other current assets was primarily due to prepayments for clinical trials and prepayments of insurance policies.

During the year ended December 31, 2014, operating activities used $7.6 million of cash, primarily resulting from our net loss of $8.5 million, partially offset by cash provided by changes in our operating assets and liabilities of $0.8 million. Net cash provided by changes in our operating assets and liabilities during the year ended December 31, 2014

consisted primarily of a $0.8 million increase in accounts payable and a $0.2 million increase in accrued expenses, partially offset by a $0.2 million increase in prepaid expenses and other current assets. The increases in accounts payable and accrued expenses were primarily due to higher clinical trial costs incurred in 2014 than in 2013 related to A-101. The increase in prepaid expenses and other current assets was primarily due to a prepayment for manufacturing scale up expenses.

During the year ended December 31, 2013, our operating activities used $4.9 million of cash, primarily resulting from our net loss of $5.2 million, partially offset by cash provided by net changes in our operating assets and liabilities of $0.3 million, which primarily consisted of an increase in accounts payable. The increase in accounts payable was primarily due to costs incurred in connection with the commencement of preclinical studies and a clinical trial of A-101 in 2013.

Investing Activities

During the year ended December 31, 2015, we used cash of $77.0 million in investing activities, consisting of purchases of marketable securities of $82.5 million and purchases of equipment of $0.5 million, partially offset by proceeds from sales and maturities of marketable securities of $6.1 million.

During the year ended December 31, 2014, we used cash of $1.8 million in investing activities, consisting of purchases of marketable securities of $5.0 million and purchases of equipment of $0.4 million, partially offset by proceeds from sales and maturities of marketable securities of $3.7 million.

Table of Contents

During the year ended December 31, 2013, we used cash of $4.5 million in investing activities, consisting of purchases of marketable securities.

Financing activities

During the year ended December 31, 2015, net cash provided by financing activities was $ 96.4 million, consisting of $39.8 million of net proceeds received from our issuance of Series C convertible preferred stock in August 2015 and net proceeds of $56.6 million received from our initial public offering of common stock in October 2015.

During the year ended December 31, 2014, net cash provided by financing activities was $10.6 million as a result of net proceeds received from our issuance of Series B convertible preferred stock in September 2014.

We had no cash flows from financing activities during the year ended December 31, 2013.

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 continue to invest in discovery efforts to explore 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.

As a publicly traded company, we have incurred and will continue to 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 were not applicable to us prior to our IPO. We expect ongoing compliance with these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

We believe our existing cash, cash equivalents and marketable securities are sufficient to fund our operating and capital expenditure requirements for a period greater than 12 months from December 31, 2015 based on our current operating assumptions, including the completion of our three ongoing 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 Phase 2 clinical trials for A-101 for the treatment of common warts. These assumptions 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 commercially acceptable 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, your ownership will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a holder of our common stock.

Table of Contents

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.

See “Risk Factors” for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2015 and the effect such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments Due by Period				
	Total	Less Than 1 Year	1 3 Years	4 5 Years	More than 5 Years
	(in thousands)				

Operating lease commitments	$ 1,019	$ 237	$ 531	$ 251	$ —
Total	$ 1,019	$ 237	$ 531	$ 251	$ —

We sublease office space in Malvern, Pennsylvania under an operating lease agreement with a term through November 2019.

Under various agreements, we will be required to make milestone payments and pay royalties and other amounts to third parties. We have not included any contingent payment obligations, such as milestones or royalties, in the table above as the amount, timing and likelihood of such payments are not known.

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.

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

Table of Contents

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.

Under a commercial license agreement with other third parties that we entered into in November 2015, we have agreed to make aggregate payments of up to $2.35 million upon the achievement of specified pre-commercialization milestones, such as clinical trials and regulatory approvals. We will pay an annual maintenance fee of $50,000, to be credited against any milestone fees or royalties paid in each calendar year. With respect to any products we commercialize under the agreement, we will pay tiered royalties at a low to mid-single-digit percentage of annual net sales, subject to specified reductions, as determined on a country-by-country and product-by-product basis, until the date that all of the patent rights for that product have expired, or in specified countries under specified circumstances, ten years from the first commercial sale of such product.

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 Securities and Exchange Commission.

Recently Issued and Adopted Accounting Pronouncements

In November 2015, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU 2015- 17, Balance Sheet Classification of Deferred Taxes. The amendments in this update simplify the presentation of deferred income taxes to require that deferred tax liabilities and assets are classified as noncurrent in a statement of financial position. The amendments are effective for annual reporting periods beginning after December 15, 2016 and interim reporting periods within those annual periods. Early application is permitted. We have adopted the provisions of ASU 2015-17 early, the impact of which on our consolidated financial statements was not significant.

In January 2016, the FASB issued ASU 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities. The amendments in this update revise the accounting related to the classification and measurement of

investments in equity securities and the presentation of certain fair value changes for financial liabilities measured at fair value. The amendments are effective for annual reporting periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted. We are currently evaluating the potential impact of the adoption of this standard.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The new standard establishes a right-of-use, or ROU, model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. The new standard is effective for annual periods beginning after December 15, 2018, including interim periods within those annual periods, with early adoption permitted. A modified retrospective transition approach is required for lessees for capital and operating leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, with certain practical expedients available. We are currently evaluating the potential impact of the adoption of this standard.

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 7A. Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risk related to changes in interest rates. Our cash equivalents and marketable securities consist of money market funds, asset-backed securities, commercial paper, corporate debt securities and government agency debt. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our marketable securities are subject to interest rate risk and will fall in value if market interest rates increase. However, due to the short-term nature and low-risk profile of our investment portfolio, we do not expect that an immediate 10% change in market interest rates would have a material effect on the fair market value of our investment portfolio. We have the ability to hold our marketable securities until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

.

Item 8. Financial Statements and Supplementary Data

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Table of Contents

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of

Aclaris Therapeutics, Inc.

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of operations and comprehensive loss, of convertible preferred stock and stockholders' (deficit) equity and of cash flows, present fairly, in all material respects, the financial position of Aclaris Therapeutics, Inc. and its subsidiaries at December 31, 2015 and 2014, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2015 in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Philadelphia, Pennsylvania

March 23, 2016

81

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share data)

	December 31, 2015	2014
Assets		
Current assets:		
Cash and cash equivalents	$ 9,851	$ 10,757
Marketable securities	75,017	5,373
Prepaid expenses and other current assets	1,656	204
Total current assets	86,524	16,334
Marketable securities	7,170	518
Property and equipment, net	360	515
Other assets	22	10
Total assets	$ 94,076	$ 17,377
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 810	$ 1,263
Accrued expenses	745	188
Total current liabilities	1,555	1,451
Deferred rent	—	4
Total liabilities	1,555	1,455
Commitments and contingencies (Note 10)		
Convertible preferred stock (Series A, B and C), $0.00001 par value; 34,090,000 shares authorized at December 31, 2014; 27,341,057 shares issued and outstanding at December 31, 2014; aggregate liquidation preference of $35,882 at December 31, 2014	—	36,677
Stockholders' Equity (Deficit):		
Preferred stock, $0.00001 par value; 10,000,000 shares authorized and no shares issued at December 31, 2015; no shares authorized at December 31, 2014	—	—
Common stock, $0.00001 par value; 100,000,000 and 77,000,000 shares authorized at December 31, 2015 and 2014, respectively; 20,157,503 and 2,730,427 shares issued and outstanding at December 31, 2015 and 2014, respectively	—	—
Additional paid in capital	135,503	—
Accumulated other comprehensive loss	(149)	(6)
Accumulated deficit	(42,833)	(20,749)
Total stockholders' equity (deficit)	92,521	(20,755)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 94,076	$ 17,377

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share data)

	Year Ended December 31, 2015	2014	2013
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	15,339	6,507	3,488
General and administrative	5,328	2,026	1,769
Total operating expenses	20,667	8,533	5,257
Loss from operations	(20,667)	(8,533)	(5,257)
Interest income	104	16	21
Net loss	(20,563)	(8,517)	(5,236)
Accretion of convertible preferred stock	(2,566)	(2,054)	(1,740)
Net loss attributable to common stockholders	$ (23,129)	$ (10,571)	$ (6,976)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.79)	$ (6.15)	$ (6.45)
Weighted average common shares outstanding, basic and diluted	6,107,042	1,720,082	1,081,347
Other comprehensive income (loss):			
Unrealized (loss) gain on marketable securities, net of tax of $0	(148)	(9)	3
Foreign currency translation adjustments	5	—	—
Total other comprehensive (loss) income	(143)	(9)	3
Comprehensive loss	$ (20,706)	$ (8,526)	$ (5,233)

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK

AND STOCKHOLDERS' EQUITY (DEFICIT)

(In thousands, except share data)

	Series A, B and C Convertible Preferred Stock		Common Stock		Additional Paid in	Accumulated Other Comprehensive Income	Accumulated	Total Stockholders' (Deficit)
	Shares	Amount	Shares	Par Value	Capital	(Loss)	Deficit	Equity
Balance at December 31, 2012	20,890,000	$ 21,260	2,730,427	$ —	$ —	$ —	$ (2,190)	$ (2,190)
Unrealized gain on marketable securities	—	—	—	—	—	3	—	3
Accretion of redeemable convertible preferred stock to redemption value	—	1,740	—	—	—	—	(1,740)	(1,740)
Net loss	—	—	—	—	—	—	(5,236)	(5,236)
Balance at December 31, 2013	20,890,000	23,000	2,730,427	—	—	3	(9,166)	(9,163)
Issuance of Series B redeemable convertible preferred stock and purchased put option, net of issuance costs of $60	6,451,057	11,623	—	—	—	—	(1,039)	(1,039)
Unrealized loss on marketable securities	—	—	—	—	—	(9)	—	(9)
Stock-based compensation	—	—	—	—	27	—	—	27

expense								
Accretion of redeemable convertible preferred stock to redemption value	—	2,054	—	—	(27)	—	(2,027)	(2,054)
Net loss	—	—	—	—	—	—	(8,517)	(8,517)
Balance at December 31, 2014	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	—	—	—	—	—	—
Issuance of common stock in connection with IPO, net of offering costs of $2,272	—	—	5,750,000	—	56,550	—	—	56,550
Issuance of common stock upon conversion of convertible preferred stock	(40,286,041)	(78,305)	11,677,076	—	78,305	—	—	78,305
Unrealized loss on marketable securities	—	—	—	—	—	(148)	—	(148)
Foreign currency translation adjustment	—	—	—	—	—	5	—	5
Stock-based compensation expense	—	—	—	—	891	—	—	891
Accretion of redeemable convertible preferred stock to redemption value	—	1,764	—	—	(243)	—	(1,521)	(1,764)
Net loss	—	—	—	—	—	—	(20,563)	(20,563)
Balance at December 31, 2015	—	$ —	20,157,503	$ —	$ 135,503	$ (149)	$ (42,833)	$ 92,521

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

Table of Contents

ACLARIS THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,		
	2015	2014	2013
Cash flows from operating activities:			
Net loss	$ (20,563)	$ (8,517)	$ (5,236)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation expense	90	12	11
Stock-based compensation expense	891	27	—
Deferred rent	(5)	1	3
Write-down of property and equipment held for sale	289	—	—
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	(1,269)	(152)	7
Accounts payable	(359)	819	303
Accrued expenses	557	174	(8)
Net cash used in operating activities	(20,369)	(7,636)	(4,920)
Cash flows from investing activities:			
Purchases of property and equipment	(507)	(417)	—
Purchases of marketable securities	(82,513)	(5,035)	(4,535)
Proceeds from sales and maturities of marketable securities	6,069	3,673	—
Net cash used in investing activities	(76,951)	(1,779)	(4,535)
Cash flows from financing activities:			
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	39,864	10,584	—
Proceeds from initial public offering, net of offering costs	56,550	—	—
Net cash provided by financing activities	96,414	10,584	—
Net (decrease) increase in cash and cash equivalents	(906)	1,169	(9,455)
Cash and cash equivalents at beginning of period	10,757	9,588	19,043
Cash and cash equivalents at end of period	$ 9,851	$ 10,757	$ 9,588
Supplemental disclosure of non-cash investing and financing activities:			
Additions to property and equipment purchases included in accounts payable	$ 2	$ 91	$ —
Accretion of convertible preferred stock to redemption value	$ 1,764	$ 2,054	$ 1,740
Fair value of preferred stock purchased put option on date of issuance	$ —	$ 1,039	$ —

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

85

Table of Contents

ACLARIS THERAPEUTICS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

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

1. Organization and Nature of Business

Aclaris Therapeutics, Inc. was incorporated under the laws of the State of Delaware in 2012. On July 17, 2015, Aclaris Therapeutics International Limited ("ATIL") was established under the laws of the United Kingdom as a wholly-owned subsidiary of Aclaris Therapeutics, Inc. (together with ATIL referred to as the "Company"). 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.

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 Company's common stock 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. The conversion of the convertible preferred stock was a non-cash transaction which has been excluded from the Consolidated Statements of Cash Flows.

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 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 underwriting discounts and commissions of $4,428 to the underwriters in connection with the IPO, including the underwriters' exercise of their option to purchase additional shares. In addition, the Company incurred expenses of $2,272 in connection with the IPO. The net offering proceeds received by the Company, after deducting underwriting discounts, commissions and offering expenses, were $56,550.

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 then-outstanding convertible preferred stock (see Note 6). Accordingly, all share and per share amounts for all periods presented in these consolidated 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 consolidated 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 December 31, 2015, the Company had cash, cash equivalents and marketable securities of $92,038 and an accumulated deficit of $42,833. 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, cash equivalents and marketable securities as of December 31, 2015 will be sufficient to fund its operations for a period greater than 12 months based on its current operating assumptions. The

Table of Contents

future 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 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, ATIL. 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, research and development expenses and the valuation of 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.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses include salaries, stock-based compensation and benefits of employees, fees paid under licensing agreements, fees paid under a third party assignment agreement and other operational costs related to the Company's research and development activities, including depreciation expenses and the cost of research and development contracts which the Company has entered into with outside vendors to conduct both preclinical studies and clinical trials. Significant judgment and estimates are made in determining the amount of research and development costs recognized in each reporting period. The Company analyzes the progress of its studies and clinical trials, completion of milestone events, invoices received and

contracted costs when estimating research and development costs. Actual results could differ from the Company's estimates. The Company's historical estimates for research and development costs have not been materially different from the actual costs.

Foreign Currency Translation

The reporting currency of the Company is the U.S. Dollar. The functional currency of ATIL, the Company's wholly-owned subsidiary, is the British Pound. Assets and liabilities of ATIL are translated into U.S. Dollars based on exchange rates at the end of each reporting period. Revenues and expenses are translated at average exchange rates during the reporting period. Gains and losses arising from the translation of assets and liabilities are included as a component of accumulated other comprehensive loss or income. Gains and losses resulting from foreign currency transactions are reflected within the Company's results of operations. The Company has not utilized any foreign currency hedging strategies to mitigate the effect of its foreign currency exposure.

Stock-Based Compensation

The Company measures the compensation expense of stock-based awards granted to employees and directors using the grant date fair value of the award and recognizes compensation expense for share-based awards expected to vest on a straight-line basis over their requisite service period, which is generally the vesting period of the respective award. The Company has issued stock options and restricted stock unit awards with service-based vesting conditions.

The Company initially measures the compensation expense of stock-based awards granted to consultants using the grant date fair value of the award. Compensation expense is recognized over the period during which services are rendered by such consultants. At the end of each financial reporting period prior to completion of services being

Table of Contents

rendered, the compensation expense is remeasured using the then current fair value of the stock-based award, based on updated assumption inputs in the Black-Scholes option pricing model.

The Company classifies stock-based compensation expense in its statement of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipients' service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre vesting forfeitures for service based awards. The impact of a forfeiture rate adjustment will be recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to stock-based compensation expense in future periods.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Prior to the IPO, the Company was a private company and lacked company specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain vanilla" options. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future. Prior to the IPO, the Company valued its common stock using a hybrid method which used market approaches to estimate enterprise value. The hybrid method used was a probability-weighted expected return method which is a scenario-based methodology that estimated the fair value of the Company's common stock based upon an analysis of future values for the Company assuming various outcomes. The hybrid method used calculated equity values using an option pricing model in one or more of scenarios, and also considered the rights of each class of stock.

The fair value of each restricted stock unit award is measured as the aggregate difference between the purchase price per share of the award, if any, and the fair value per share of the Company's common stock on the date of grant.

Patent Costs

All patent related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more likely than not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the

Table of Contents

effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Accretion of Convertible Preferred Stock

Accretion of convertible preferred stock includes the accretion of accruing dividends on and issuance costs of the Company's Series A, B and C convertible preferred stock. The carrying values of the Series A and Series B convertible preferred stock were accreted to their respective redemption values, using the effective interest method, from the date of issuance through August 28, 2015. In connection with the closing of the Company's Series C convertible preferred stock financing on August 28, 2015, the redemption rights of the Series A and B convertible preferred stock were removed. Subsequent to August 28, 2015, the Company was no longer required to record the accumulated undeclared dividends on its balance sheet, but was thereafter required to deduct accumulated undeclared dividends as part of its per share calculation (see Note 6). On October 13, 2015, in connection with the Company's IPO, all of the Company's convertible preferred stock was converted to common stock.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. Comprehensive loss is comprised of net loss, foreign currency translation adjustments and unrealized gains (losses) on marketable securities.

Net Loss per Share

Basic net loss per share is computed using the weighted average number of common shares outstanding during the period. Diluted net loss per share is computed using the sum of the weighted average number of common shares outstanding during the period and, if dilutive, the weighted average number of potential shares of common stock, including the assumed exercise of stock options and unvested restricted stock. Prior to the IPO, the Company applied the two-class method to calculate its basic and diluted net loss per share attributable to common stockholders, as its convertible preferred stock and common stock are participating securities. The two-class method is an earnings allocation formula that treats a participating security as having rights to earnings that otherwise would have been available to common stockholders. However, the two-class method does not impact the net loss per share of common stock as the Company was in a net loss position for each of the periods presented and preferred stockholders do not participate in losses.

The Company's convertible preferred stock contractually entitles the holders of such shares to participate in dividends but does not contractually require the holders of such shares to participate in losses of the Company. Similarly, restricted stock awards granted by the Company entitle the holder of such awards to dividends declared or paid by the board of directors, regardless of whether such awards are unvested, as if such shares were outstanding common shares at the time of the dividend. However, the unvested restricted stock awards are not entitled to share in the residual net assets (deficit) of the Company. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Cash Equivalents

The Company considers all short term, highly liquid investments with original maturities of 90 days or less at acquisition date to be cash equivalents. Cash equivalents, which consist of money market accounts and corporate debt securities with original maturities of less than three months, are stated at fair value.

Marketable Securities

Marketable securities with original maturities of greater than three months and remaining maturities of less than one year from the balance sheet date are classified as short term. Marketable securities with remaining maturities of greater than one year from the balance sheet date are classified as long term.

Table of Contents

The Company classifies all of its marketable securities as available-for-sale securities. The Company's marketable securities are measured and reported at fair value using quoted prices in markets that are not active for identical or similar securities. Unrealized gains and losses are reported as a separate component of stockholders' equity (deficit). The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense) within the statement of operations and comprehensive loss. If any adjustment to fair value reflects a decline in the value of the investment, the Company considers available evidence to evaluate the extent to which the decline is "other than temporary" and reduces the investment to fair value through a charge to the statement of operations and comprehensive loss.

Assets Held for Sale

In order for an asset to be classified as held for sale, several criteria must be achieved. These criteria include, among others, an active program to market an asset and locate a buyer, as well as the probable disposition of the asset within one year. Upon being classified as held for sale, the recoverability of the carrying value of an asset must be assessed and evaluated. After the valuation process is completed, the held for sale asset is reported at the lower of its carrying value or fair value less cost to sell, and no additional depreciation expense is recognized related to the asset. Once an asset is classified as held for sale, all of its historical balance sheet information is included in prepaid expenses and other current assets in the accompanying consolidated balance sheets. During the year ended December 31, 2015, the Company determined that several pieces of machinery used in the Company's scale-up operations would no longer be part of the Company's future operations. The Company engaged a third-party to market the assets and locate a buyer in the fourth quarter of 2015, and believes it is probable that the sale of the assets will occur sometime in 2016. The Company utilized the third-party seller's estimate of the fair value of the machinery. During the year ended December 31, 2015, the Company recorded an impairment charge of $289 to write the assets down to their net realizable value. The impairment charge is included in research and development expense on the Company's consolidated statement of operations and comprehensive loss. As of December 31, 2015, $216 in assets were classified as held for sale.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

· Level 1 — Quoted prices in active markets for identical assets or liabilities.

- Level 2 — Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.

- Level 3 — Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy described above. The carrying value of the Company's accounts payable and accrued expenses approximate fair value due to the short-term nature of these liabilities.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and marketable securities. The Company holds all cash, cash equivalents and marketable securities balances at one accredited financial institution, in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

Table of Contents

The Company is dependent on third party manufacturers to supply products for research and development activities of its programs, including preclinical and clinical testing. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and other components.

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' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. The Company did not record any deferred offering costs as of December 31, 2015 or 2014.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. Computer equipment is depreciated over three years. Manufacturing equipment is depreciated over five years. Furniture and fixtures are depreciated over seven years. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations.

Impairment of Long Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is identifying, developing and commercializing innovative and differentiated drugs to address significant unmet needs in dermatology. No revenue has been generated since inception, and all tangible assets are held in the United States.

Recently Issued and Adopted Accounting Pronouncements

In November 2015, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2015- 17, Balance Sheet Classification of Deferred Taxes. The amendments in this update simplify the presentation of deferred income taxes to require that deferred tax liabilities and assets are classified as noncurrent in a statement of financial position. The amendments are effective for annual reporting periods beginning after December 15, 2016 and interim reporting periods within those annual periods. Early application is permitted. The Company has adopted the provisions of this standard early, the impact of which on its consolidated financial statements was not significant.

In January 2016, the FASB issued ASU 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities. The amendments in this update revise the accounting related to the classification and measurement of investments in equity securities and the presentation of certain fair value changes for financial liabilities measured at fair value. The amendments are effective for annual reporting periods beginning after December 15, 2017, including

Table of Contents

interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the potential impact of the adoption of this standard.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The new standard establishes a right-of-use ("ROU") model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. ASU 2016-02 is effective for annual periods beginning after December 15, 2018, including interim periods within those annual periods, with early adoption permitted. A modified retrospective transition approach is required for lessees for capital and operating leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, with certain practical expedients available. The Company is currently evaluating the potential impact of the adoption of this standard.

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 December 31, 2015 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 8,810	$ 250	$ —	$ 9,060
Marketable securities	—	82,187	—	82,187
Total Assets	$ 8,810	$ 82,437	$ —	$ 91,247

	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 December 31, 2015, the Company's cash equivalents consisted of money market funds and corporate debt securities with original maturities of less than three months. As of December 31, 2014, the Company's cash equivalents consisted of money market funds. The Company valued its money market funds based on Level 1 inputs. In determining the fair value of its corporate debt securities, commercial paper, asset-backed securities and U.S. government agency debt securities as of December 31, 2015 and 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 years ended December 31, 2015 and 2014, there were no transfers between Level 1, Level 2 and Level 3.

Table of Contents

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

	December 31, 2015			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Marketable securities:				
Corporate debt securities	$ 46,270	$ —	$ (125)	$ 46,145
Commercial paper	9,789	—	—	9,789
Asset-backed securities	6,234	—	(14)	6,220
U.S. government agency debt securities	20,048	—	(15)	20,033
Total marketable securities	$ 82,341	$ —	$ (154)	$ 82,187