AMBIT BIOSCIENCES CORP
Form 10-K
March 20, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

Form 10-K

(Mark One)

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

For the fiscal year ended December 31, 2013

or

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

For the transition period from to

Commission file number: 001-35919

Ambit Biosciences Corporation

(Exact Name of Registrant as Specified in its Charter)

Delaware	**33-0909648**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
11080 Roselle St., San Diego CA	**92121**
(Address of Principal Executive Offices)	**(Zip Code)**

(858) 334-2100

(Registrant s Telephone Number, Including Area Code)

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

Title of Each Class	**Name of Each Exchange on Which Registered**
Common Stock, par value $0.001 per share	**The NASDAQ Stock Market LLC**

Securities registered pursuant to Section 12(g) of the Act: None

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

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

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate 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 x No ¨

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller reporting company. See 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 ¨ Smaller reporting company x

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

As of June 30, 2013, the aggregate market value of the voting stock held by non-affiliates of the registrant, computed by reference to the last sales price of such stock as of such date on the NASDAQ Stock Market LLC, was approximately $34,700,000. Excludes an aggregate of 12,919,850 shares of common stock held by officers and directors and by each person known by the registrant to over 5% or more of the outstanding common stock as of June 30, 2013. Exclusion of shares held by any of these persons should not be construed to indicate that such person possesses the power, direct or indirect, to direct or cause the direction of the management or policies of the registrant, or that such person is controlled by or under common control with the registrant.

The number of outstanding shares of the registrant s common stock, par value $0.001 per share, as of February 28, 2014 was 17,921,747.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant s definitive Proxy Statement to be filed with the Securities and Exchange Commission pursuant to Regulation 14A in connection with the registrant s 2014 Annual Meeting of Stockholders, which will be filed subsequent to the date hereof, are incorporated by reference into Part III of this Form 10-K. Such Proxy Statement will be filed with the Securities and Exchange Commission not later than 120 days following the end of the registrant s fiscal year ended December 31, 2013.

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AMBIT BIOSCIENCES CORPORATION

FORM 10-K

For the Fiscal Year Ended December 31, 2013

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PART I

Forward-Looking Statements

This Annual Report on Form 10-K may contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, which represent our intent, belief, or current expectations, involve risks and uncertainties. We use words such as may, will, expect, anticipate, estimate, intend, plan, predict, potential, believe, should and similar expressions to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements may include, but are not limited to, statements concerning: (i) the initiation, cost, timing, progress and results of our research and development activities, preclinical studies and future clinical trials, including our expected timeline for nominating clinical development candidates under our strategic alliances and our expected timeline for filing applications with regulatory authorities;(ii) our ability to obtain and maintain regulatory approval of our future product candidates, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate; (iii)our ability to obtain funding for our operations; (iv)our plans to research, develop and commercialize our future product candidates; (v)our ability to attract collaborators with development, regulatory and commercialization expertise; (vi)our ability to obtain and maintain intellectual property protection for our future product candidates; (vii) the size and growth potential of the markets for our future product candidates, and our ability to serve those markets; (viii)our ability to successfully commercialize our future product candidates; (ix)the rate and degree of market acceptance of our product candidates; (x)our ability to develop sales and marketing capabilities, whether alone or with potential future collaborators; (xi)regulatory developments in the United States and foreign countries; and (xii)the performance of our third-party suppliers and manufacturers. Although we believe the expectations reflected in these forward-looking statements are reasonable, such statements are inherently subject to risk and we can give no assurances that our expectations will prove to be correct. You should not place undue reliance on these forward-looking statements, which apply only as of the date of this Annual Report on Form 10-K. As a result of many factors, including without limitation those set forth under Risk Factors under Item 7 of Part II of this annual report on Form 10-K, our actual results may differ materially from those anticipated in these forward-looking statements. We undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Item 1. Business.

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of drugs to treat unmet medical needs in oncology, autoimmune and inflammatory diseases by inhibiting kinases that are important drivers for those diseases. Our pipeline currently includes three programs, each discovered internally and each aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, is a once-daily, orally administered FMS-like tyrosine kinase 3, or FLT3, kinase inhibitor. We are planning to initiate a Phase 3 clinical trial in patients with relapsed/refractory acute myeloid leukemia, or AML, who express a genetic mutation in FLT3, in the second quarter of 2014.

We believe there is a significant unmet need for more effective treatments of AML, particularly for the subset of patients expressing a genetic mutation in FLT3, known as the FLT3 internal tandem duplication, or FLT3-ITD, mutation. The FLT3-ITD mutation acts like a power switch that causes leukemic cells, or blasts, to spread more aggressively and grow back more rapidly following chemotherapy, conferring an especially poor survival outcome.

Quizartinib is designed to turn off this switch. Our initial regulatory strategy for quizartinib is focused on relapsed/refractory AML. However, we plan to also develop quizartinib in other AML therapeutic settings, such as a frontline therapy in newly diagnosed AML patients in combination with chemotherapy, followed by continuous quizartinib maintenance therapy, including quizartinib maintenance following a hematopoietic stem cell transplant, or HSCT.

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We have studied quizartinib in over 480 patients in Phase 1 and 2 clinical trials. Our Phase 2 and Phase 2b clinical trials demonstrated the following key clinical benefits of quizartinib as a monotherapy: a high response rate in relapsed/refractory FLT3-ITD positive patients; a substantial number of patients who were bridged to a potentially curative HSCT, commonly referred to as a bone marrow transplant; and median overall survival in FLT3-ITD positive patients which compared favorably to historical survival data reported for both FLT3-ITD positive AML patients.

Our second drug candidate in clinical development, AC410, is a potent, selective, orally-administered, small molecule inhibitor of Janus kinase 2, or JAK2, that has potential utility for treatment in oncology, autoimmune and inflammatory diseases. Signaling through JAK controls the activation, proliferation and survival of various types of immune cells, and over-activation of such cells can exacerbate a variety of normal inflammatory processes, resulting in inflammation, or promotion of more aggressive neoplastic disease. Our initial JAK2 drug candidate, AC430, is a racemic mixture (50/50) of two enantiomers (mirror images), AC410 and AC409, and was studied in a Phase 1 clinical trial. We plan to advance AC410 to proof-of-concept clinical trials in one or more oncology, autoimmune and/or inflammatory diseases, independently or in collaboration with a strategic partner.

Our third program consists of a potent and selective small molecule compound, AC708, which inhibits the colony-stimulating factor-1 receptor, or CSF1R, a receptor tyrosine kinase. Signaling through CSF1R controls the activation, proliferation and survival of macrophages, which are key mediators of immune system function. Over-activation of macrophages may result in exacerbation of certain diseases, including oncology, autoimmune and inflammatory diseases. We are expanding our pre-clinical studies of AC708 in oncology disease models to better define our plans for our Phase 1 and Phase 2 clinical trials. We plan to develop AC708 independently or in collaboration with a strategic partner.

Our Strategy

The key components of our strategy are:

Develop and seek regulatory approval for our lead drug candidate, quizartinib, in relapsed/refractory AML patients who express a genetic mutation in FLT3.

Expand the clinical development of quizartinib in AML and other hematological disease indications.

Pursue strategic partnerships outside North America to accelerate development of quizartinib in indications beyond relapsed/refractory AML and expand the commercial opportunity for quizartinib.

Maximize strategic value by establishing a commercial capability to market, sell and distribute quizartinib in North America.

Advance the development of our JAK2 and CSF1R programs through a combination of internal development and strategic partnerships.

Leverage our core competency and proprietary chemical library to continue the discovery and development of a broad pipeline of novel drug candidates that inhibit validated kinase targets to address diseases with unmet medical need.

Background

Kinases are a family of over 500 enzymes that play essential roles in signaling and regulation of important cellular processes such as activation, growth, proliferation, differentiation and survival. This key role in regulating the life cycle of cells also means that kinases can be involved in the underlying mechanisms for many human diseases, including oncology, autoimmune and inflammatory diseases. Kinases have proven to be a rich source of targets for drug development with 23 approved drugs in oncology and inflammatory disease since 2001. Our core competency is the discovery, optimization and development of highly selective and potent, orally-available small molecule drug candidates that inhibit validated kinase targets in diseases with significant unmet medical need. We have built our pipeline using our proprietary compound library designed specifically to inhibit kinases and expect to continue to leverage this library to develop viable drug candidates in the future.

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Quizartinib an Oral FLT3 Inhibitor for AML

Background of Current Treatment for AML

AML is the most common type of acute leukemia in adults and is projected to account for approximately 36% of all new leukemia cases in 2014. AML results in uncontrolled growth and accumulation of malignant white blood cells which fail to function normally and interfere with the production of normal blood cells. According to the American Cancer Society, approximately 18,860 patients will be newly diagnosed with AML in 2014 in the United States and approximately 10,460 are expected to die of the disease in 2014.

AML is generally a disease of older people and the median age of a patient at initial diagnosis is 66 years. The five-year survival rate for all AML patients, irrespective of age and FLT3-ITD status, is 23%.

The standard of care for AML has not changed appreciably for decades. The goal of treatment in AML is to reduce the blasts in the bone marrow to below 5% and return the blood cell counts to near normal levels. This is considered a complete remission, or CR. Variations of CR include CRi, which is a complete remission with incomplete neutrophil recovery with or without incomplete platelet recovery; and CRp, which is a complete remission with incomplete platelet recovery. The composite complete response (CRc) is the addition of CR + CRp + CRi. An HSCT is generally recognized as the only curative treatment option. Typically, patients who are able to achieve a reduction in bone marrow blasts below 5% are more suitable candidates for an HSCT and have an improved projected outcome following an HSCT.

Role of FLT3 in AML and the Need for New Therapies for AML Patients with FLT3-ITD mutation

AML is a particularly aggressive and deadly disease, especially for patients with the FLT3-ITD mutation. FLT3 is a kinase receptor expressed on hematopoietic progenitor cells (immature blood cells) and plays a critical role in regulating their activation, growth, proliferation, survival and differentiation into mature blood cells. Over 35% of AML patients over age 55 are estimated to harbor the FLT3-ITD mutation. The FLT3-ITD mutation results in aggressive proliferation of immature, irregular blasts that lack the ability to differentiate into normal blood cells. Patients who harbor the FLT3-ITD mutation are known as FLT3-ITD positive, and have a significantly worse prognosis compared to FLT3-ITD negative patients. FLT3-ITD positive patients typically respond to induction chemotherapy; however, they tend to relapse more quickly and at a higher rate, leading to an overall survival rate that is much lower than FLT3-ITD negative patients.

Quizartinib Development Strategy: Seek Initial Approval in Relapsed/Refractory AML

We believe that there is a significant unmet need for an approved agent that offers a targeted and effective approach to AML and that can be used either as monotherapy or in combination with chemotherapy. In addition, we believe there is a need for a better tolerated and more convenient therapy which can be used across multiple patient populations and settings, including younger and elder patients, newly diagnosed and relapsed/refractory patients, and patients that have undergone an HSCT.

We have completed Phase 2 and 2b clinical trials in relapsed/refractory AML patients and results from these trials were reported at the 2012 Annual Meeting of the American Society of Hematology, or ASH, the 2013 Annual Meeting of the American Society of Clinical Oncology, or ASCO and the 2013 Annual Meeting of ASH. These clinical trials demonstrated the following three key clinical benefits of quizartinib:

1. At multiple doses, quizartinib as a monotherapy demonstrated a high response rate in relapsed/refractory FLT3-ITD positive patients;

2. A substantial number of patients treated with quizartinib responded to therapy and were bridged to a potentially curative HSCT resulting in better overall survival compared to responders that did not undergo an HSCT; and

3. Overall survival in FLT3-ITD positive patients treated with quizartinib compared favorably to historical survival data reported for FLT3-ITD positive AML patients.

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Based on these results our initial development strategy for quizartinib is to seek approval in relapsed/refractory AML patients with the FLT3-ITD mutation.

Phase 3 Clinical Trial

We plan to initiate a registrational Phase 3 clinical trial in relapsed/refractory AML patients with the FLT3-ITD mutation in the second quarter of 2014. This Phase 3 clinical trial will compare quizartinib as monotherapy to one of three chemotherapy regimens (MEC, Flag-Ida or LoDAC) as determined by the physician with a 2:1 randomization of quizartinib to chemotherapy. The trial will be conducted in FLT3-ITD positive patients over the age of 18 who have relapsed from, or are refractory to, frontline chemotherapy, including those patients relapsing following an HSCT. Dosing will be initiated at 30 mg daily, with the potential for dose escalation to 60 mg at day 16 or after day 28. Patients will be dosed continually until disease progression or intolerable toxicity. Patients who proceed to HSCT after quizartinib treatment will have the option to reinitiate treatment with quizartinib following the transplant. The trial is expected to enroll approximately 326 patients in the United States, Western Europe, Canada and Australia. The primary endpoint for the Phase 3 clinical trial will be overall survival. An interim analysis will be conducted, and will include an adaptive design component that will allow the Data Safety Monitoring Board, or DSMB, to increase the number of patients if warranted. Enrollment is expected to be completed in the second half of 2015 assuming there is no increase in the number of patients following the interim analysis.

Phase 2 Clinical Trial Results

Results of our multi-center, open label Phase 2 clinical trial of quizartinib as monotherapy in relapsed/refractory AML patients were reported at the ASH Annual Meeting in December of 2012 and at the ASCO Annual Meeting in June of 2013. This clinical trial was designed to evaluate the efficacy and safety of quizartinib in relapsed/refractory AML patients both with and without the FLT3-ITD mutation, with 333 patients enrolled at clinical sites in the United States, Canada, and select European countries (France, Germany, Italy, Netherlands, Spain, United Kingdom and Poland). A total of 248 FLT3-ITD positive patients with relapsed/refractory AML and 84 patients without the FLT3-ITD mutation were enrolled. One patient had an unknown FLT3-ITD status at time of enrollment. Multiple doses were explored in this study, including 200 mg/day (17 patients), 135 mg/day (166 patients) and 90 mg/day (150 patients). This clinical trial enrolled two distinct patient populations:

- Cohort 1 focused on elderly patients who were ³ 60 years of age who relapsed after one first-line chemotherapy regimen and who were either in complete remission of less than 12 months or were primary refractory to first-line chemotherapy treatment.

- Cohort 2 focused on patients who were, on average, younger and had received more extensive prior therapy than those enrolled in Cohort 1, and included patients who were ³ 18 years of age (this includes patients ³ 60 years of age) who were relapsed or refractory after one second-line (salvage)-chemotherapy regimen or were relapsed or refractory after an HSCT.

Patients enrolled in this trial had a median blast count at initial enrollment of 80%. The composite complete response (CRc) rate in FLT3-ITD positive patients in the Phase 2 clinical trial was 50% (125/248 patients) with median time for such patients to achieve a CRc of 4.3 weeks. Additionally 25% (62/248 patients) of the FLT3-ITD positive patients in the Phase 2 trial achieved a partial response. A partial response (PR) is defined as reduction of blasts to between 25% and 5% with at least a 50% reduction from baseline.

In relapsed/refractory AML patients, the objective of treatment is achieving remission through a significant reduction in blasts and then, if available, proceeding to an HSCT. An HSCT remains the only recognized potential cure for relapsed/refractory AML patients and therefore proceeding to or bridging to an HSCT is a key objective in the treatment of patients who are healthy enough to undergo the procedure. Because patients who are refractory to or have relapsed after one or more prior lines of treatment do not typically achieve remission from additional therapy, very few relapsed/refractory AML patients are eligible for an HSCT with the currently approved therapies for AML.

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Of the 248 FLT3-ITD positive patients in our Phase 2 clinical trial, 11 out of 112 (9.8%) patients in Cohort 1 and 47 out of 136 (34.6%) patients in Cohort 2 were bridged to an HSCT. We believe that the significantly higher number of patients who bridged to an HSCT in Cohort 2 is due to the fact that these patients were, on average, younger than patients in Cohort 1, making them more likely to be eligible for an HSCT. For patients who responded to quizartinib and were able to undergo an HSCT, our data suggests an improvement in overall survival compared to responders that do not go to HSCT and compared to to patients that did not achieve a response to quizartinib (see Figure 1).

In Cohort 2 (patients relapsed or refractory after two lines of therapy or who were relapsed after a HSCT), the median overall survival in the FLT3-ITD positive patients was 24.0 weeks (approximately 6.0 months). This specific patient population typically confers a particularly poor survival outcome and overall there is very little published literature on AML patients in their third line of therapy. In a 2005 publication in *Cancer* on a study conducted by researchers in the Department of Leukemia, University of Texas M.D. Anderson Cancer Center, of 594 AML patients (both FLT3-ITD positive and negative) treated from 1980-2004 undergoing their third line of treatment, the median overall survival for patients was 1.5 months.

Within Cohort 2, the one-year survival rate for patients who were bridged to an HSCT following treatment with quizartinib was 36.2% (17 out of 47 patients remained alive over one year). In addition, the median overall survival was 34.1 weeks in those who responded to quizartinib and were bridged to an HSCT compared to a median overall survival of 24.1 weeks in those patients who responded but did not undergo an HSCT after treatment with quizartinib (see Figure 1).

Figure 1: Cohort 2 Overall Survival for FLT3-ITD Positive Patients Who Were Bridged (N=47) to an HSCT Compared to Those Who Were Unable to Receive an HSCT (N=89)

In addition, for patients who cannot undergo an HSCT, we believe that the high level of blast reduction achieved with treatment with quizartinib correlates with improved survival and with improved quality of life, especially given that quizartinib is given orally as an outpatient therapy.

FLT3-ITD positive patients in Cohort 1 (elderly and relapsed after one first-line chemotherapy treatment or who were refractory to first-line chemotherapy treatment) had a median overall survival of 25.4 weeks. As a comparison, in a 2009 report in *Leukemia Research* on a study conducted by researchers in the Department of Leukemia, University of Texas M.D. Anderson Cancer Center of 109 patients who were FLT3-ITD positive treated from 1995-2004, the median overall survival for patients who were relapsed after their first therapy was 13.0 weeks.

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In addition, as shown in Figure 2 below, in patients who were available for response evaluation at day 28 of their first treatment cycle, the median overall survival was 31.1 weeks if the patient achieved a PR or CRc to quizartinib, compared to a median overall survival of 12.9 weeks in those who did not achieve at least a PR to quizartinib. To reduce bias against the patients who did not respond with a CRc or PR, Figure 2is based on 106 patients who lived long enough to have a response assessment (at least 28 days) out of the total of 112 FLT3-ITD positive patients in Cohort 1. See Figure 2 below.

Figure 2: Cohort 1 Overall Survival for FLT3-ITD Positive Patients Who Were Available for Response at Day 28 and Who Responded to Quizartinib (N=84) Compared to Patients Who Were Available for Response at Day 28 and Who Did Not Respond to Quizartinib (N=22)

Of the total 333 patients (both FLT3-ITD positive and negative) in the Phase 2 clinical trial, 59 (17.7%) patients had an overall survival of greater than 12 months. Nearly 73% (43/59; 72.8%) were FLT3-ITD positive. Of the 59 long-term survivors, 22 patients (37.3%) were from Cohort 1and 37 patients (62.7%) were from Cohort 2, of which 26/37 (70.3%) underwent an HSCT. The median duration of treatment for the patients who had an overall survival of greater than 12 months was 46.5 weeks (range 5.3-77.1 weeks) for Cohort 1 and 10.0 weeks (range 3.3-108+weeks) for Cohort 2.

An updated analysis performed in January 2014 showed there was one patient who has remained on quizartinib treatment for nearly four years to date, and a total of 22 patients who remained alive and continued in long-term follow-up. Of the 22 patients (16 FLT3-ITD positive and 6 FLT3-ITD negative) in long term follow-up, four were treated in Cohort 1 and 18 were treated in Cohort 2. Seventeen of these 22 patients received an HSCT after quizartinib treatment. As of the January 2014 data analysis, the 22 patients had remained alive for a median of 126.4 weeks (range 84.4+ to 167.3+ weeks), which correlates to 120.1 weeks (range 84.4+ to 167.3+) in the 16 FLT3-ITD positive subjects, and 132.6 weeks (range 126.0+ to 141.1+) in the 6 FLT3-ITD negative subjects.

Current development plans for quizartinib are focused primarily on those AML patients harboring the FLT3-ITD mutation; however, responses were also seen in FLT3-ITD negative patients in our Phase 2 clinical trial. A total of 84 FLT3-ITD negative patients were enrolled in the Phase 2 trial, and a CRc was achieved in 28/84 (33.3%) of those patients. Additionally, one patient (2.3%) in Cohort 1, and 14 patients (35%) in Cohort 2 were bridged to an HSCT. Based on these observed results, it appears that the patient population eligible for treatment with quizartinib could be expanded beyond those patients harboring the FLT3-ITD mutation, and could represent on opportunity for further development.

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Phase 2b Clinical Trial Demonstrates Improved Safety, Comparable Efficacy at Lower Doses

Our multi-center, open label Phase 2b clinical trial of quizartinib as monotherapy in relapsed/refractory AML patients randomized patients into either a 30mg (n=38) or 60mg (n=38) dose. This Phase 2b clinical trial was designed to demonstrate that reducing the dose of quizartinib from those doses studied in the Phase 2 clinical trial would maintain efficacy while improving safety. This trial enrolled FLT3-ITD positive patients who were ³18 years of age who were relapsed or refractory after one second-line (salvage) chemotherapy regimen or were relapsed or refractory after an HSCT, which was the same patient population as Cohort 2 in our Phase 2 clinical trial. The primary endpoints of the Phase 2b clinical trial were CRc rate and rate of Grade 2 or higher QT prolongation. The secondary endpoints included bridge to an HSCT, CR rate, duration of remission, and overall survival. A total of 76 FLT3-ITD positive patients with relapsed/refractory AML were enrolled at clinical sites in the United States and select European countries (France, Germany, Italy and the United Kingdom). Results of our Phase 2b clinical trial were reported at the ASH Annual Meeting in December 2013.

In the Phase 2b clinical trial, the CRc rate was 47% at both quizartinib doses. The overall response rate (CRc+PR) was 61% at 30 mg and 71% at 60 mg of quizartinib administered daily. The median time to CRc was 4.4 weeks at a quizartinib dose of 30 mg, 4.6 weeks (95% CI: 4.1 to 8.0 weeks) at 60 mg, and 4.5 weeks (95% CI: 4.3 to 6.6 weeks) for all patients. Across the quizartinib Phase 2 program, duration of response was censored at the time of discontinuing treatment to proceed to an HSCT.

Patients enrolled in this trial had a median blast count at initial enrollment of 67%. Of the total 76 patients in the Phase 2b clinical trial, 28 patients (37%), including 12 of 38 patients (32%) receiving 30 mg and 16 of 38 patients (42%) receiving 60 mg, underwent HSCT immediately following quizartinib treatment. Of the 28 patients who underwent an HSCT, 23 (82%) achieved either a CRc (n = 19) or a PR (n = 4) to quizartinib prior to proceeding to a HSCT. The findings in terms of bridge to HSCT in the Phase 2b clinical trial are consistent with findings in the same patient population (i.e. Cohort 2) from the Phase 2 clinical trial in which 35% of the FLT3-ITD positive patients were bridged to an HSCT after treatment with quizartinib.

Median overall survival in the Phase 2b clinical trial was 20.7 weeks for patients treated at a starting dose of 30 mg and 25.4 weeks for patients treated at a starting dose of 60 mg. At the time of the data analysis in May 2013, thirty-seven patients (49%) remained censored for overall survival (2 randomized but not treated and not followed for survival; and 35 patients remained alive). Of the 35 patients who remained alive, there were 24 patients alive >24 weeks and 2 alive >36 weeks.

One of the significant clinical benefits of quizartinib identified in the Phase 2 clinical trial is the achievement of long term survivors (i.e. patients alive for >12 months (52 weeks)). In the Phase 2b clinical trial, the median follow-up was shorter than the Phase 2 clinical trial, yet 35 of the 76 patients (46%) remained alive (range: 7.4 40.4+) at the time of the data analysis, with 24 patients alive >24 weeks and 2 alive >36 weeks (37.9+ weeks, and 40.4+ weeks). Given these findings, with additional follow up since May of 2013, there remains the potential for additional long term survivors from quizartinib treatment in the Phase 2b clinical trial.

The Phase 2b clinical trial demonstrated comparable efficacy across both dose cohorts (30 mg and 60 mg) for the co-primary endpoint of CRc, and secondary endpoint of overall survival as shown in Table 1 below, to the efficacy and overall survival observed at the higher doses in our Phase 2 clinical trial. The observed rates of Grade 2 or higher QT prolongation decreased at the lower doses in our Phase 2b clinical trial as compared to the higher doses studied in the Phase 2 clinical trial. Based on data from our Phase 2 and Phase 2b clinical trials, we believe the efficacy at both 30 mg and 60 mg is comparable and we selected a starting dose of 30mg daily for our Phase 3 clinical trial, with potential escalation to 60mg daily.

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Table 1: Summary Efficacy Findings in the Phase 2 and Phase 2b Clinical Trials in Patients that are FLT3-ITD Positive (Total n = 324 patients)

Study	**Phase 2 Cohort 1**			**Phase 2 Cohort 2**			**Phase 2b**	
Quizartinib Dose	**90mg (N =53)**	**135 mg (N =54)**	**200mg (N=5)**	**90 mg (N = 57)**	**135 mg (N = 67)**	**200 mg (N = 12)**	**30 mg (N = 38)**	**60 mg (N = 38)**
CRc rate	**57%**	**56%**	**60%**	**47%**	**45%**	**42%**	**47%**	**47%**
PR rate	19%	20%	40%	25%	28%	50%	14%	24%
Median OS (weeks) [95% CI]	24.0 (18.3, 29.7)	25.4 (23.7, 34.1)	24.1 (10.3, 91.7)	23.3 (19.9, 32.1)	24.1 (19.3, 29.9)	23.1 (15.0, 28.9)	20.7 (16.3, 31.8)	25.4 (1.6, 42.7)

NR = Not reached; CI=Confidence interval

Safety

To date, the clinical development program for quizartinib includes over 480 patients treated in our Phase 1, Phase 2 and Phase 2b clinical trials. The adverse events that we have observed to date are manageable and the most common all grade treatment-emergent adverse events (reported in ³ 20% of subjects) included gastrointestinal toxicities (nausea and vomiting), febrile neutropenia (fever with reduction in white blood cell count), fatigue, pyrexia (fever), anemia, QT prolongation (changes in the patient s electrocardiogram pattern), edema peripheral (swelling of legs) and dysgeusia (distortion of the sense of taste). Other than the rate of QT prolongation, which decreased at lower doses, there were no major differences between safety findings in the Phase 1, Phase 2 and Phase 2b clinical trials.

QT prolongation is a common adverse event associated with multiple other kinase inhibitors and is possibly considered a class effect. The majority of cases of QT prolongation with quizartinib are asymptomatic, and occur within the first month of treatment. Additionally, the majority of patients that experienced QT prolongation did not discontinue quizartinib due to this adverse event. To date, there has been one case of Grade 4 QT interval prolongation with Torsade de pointes (an abnormal cardiac rhythm) in a female patient taking quizartinib (90mg) with multiple concomitant medications. This event resolved after quizartinib discontinuation.

In the Phase 2b clinical trial, determination of the rate of Grade 2 or greater prolongation of QT prolongation at both 30 mg and 60 mg of quizartinib was the co-primary endpoint. The QT prolongation was Grade 2 or greater in 11% of patients (4/38) at 30 mg and in 17% of patients (6/36) at 60 mg. In contrast to the low rates of Grade 2 or higher QT prolongation in the Phase 2b study, in the 333 patients in the Phase 2 clinical trial, the QT prolongation rate was Grade 2 or greater in 71% of subjects at the starting dose of 200 mg, 36% at the starting dose of 135 mg, and 43% at the starting dose of 90 mg. The Grade 3 QT prolongation rate was also seen with a much lower frequency in the Phase 2b clinical trial at both doses (3% at the starting dose of 60mg and 5% at the starting dose of 30mg) compared to that reported at the higher doses, 35% at the starting dose of 200 mg, 15% at 135 mg, and 17% (which included a single case of Grade 4 QT prolongation that resolved after quizartinib discontinuation) at 90 mg. Overall, across both Phase 2 studies in over 400 patients, the majority of maximum QT rates are Grade 0 or Grade 1. In addition, the majority of maximum changes in QT from baseline are £60 ms. There is a clear dose dependency given that the highest Grade 2 or greater rates, highest Grade 3 rates, and greatest mean maximum change from baseline in QT occurred at the highest dose of 200 mg studied in the Phase 2 clinical trial. Each of these values have decreased substantially with the

lower doses studied in the Phase 2b clinical trial.

Quizartinib Development Strategy: Market Expansion

In addition to seeking approval for the treatment of relapsed/refractory AML with quizartinib, we plan to pursue an expanded development program in newly-diagnosed AML patients and as maintenance therapy following remission or an HSCT. Numerous company and investigator sponsored trials are currently evaluating

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the use of quizartinib in combination with chemotherapy and as a maintenance therapy for patients achieving remission, including those who have received an HSCT. While response rates tend to be higher for newly diagnosed AML patients compared to relapsed/refractory, a significant number will eventually relapse. A clear unmet need remains to potentially decrease the rate of relapse after frontline treatment, and we believe quizartinib treatment in combination with the current standard of care could potentially benefit patients in the frontline setting.

Phase I Clinical Trial of Quizartinib in Combination with Chemotherapy for Frontline Therapy

We have conducted a Phase 1 dose-escalating clinical trial evaluating quizartinib in combination with standard induction and consolidation chemotherapy in newly diagnosed AML patients, as well as quizartinib maintenance after consolidation chemotherapy for patients between the ages of 18 and 60 years of age with newly diagnosed AML, irrespective of FLT3-ITD status. Results of this Phase 1 study were presented at the 2013 Annual Meeting of ASH. We believe that this trial demonstrates for the first time that quizartinib can be safely administered with induction and/or consolidation chemotherapy in newly diagnosed younger patients with AML. The patients were given cytarabine 200 mg/m^2 x 7 days and daunorubicin 60 mg/ m^2 x 3 days (7+3) for induction and high dose cytarabine 3 g/ m^2 (HiDAC) every12 hours on days 1, 3, and 5 for consolidation. Quizartinib was administered daily for either 7 or 14 days, starting at Day 4 of induction and/or consolidation chemotherapy. Enrollment of 18 total patients, across 3 dose levels were treated. There were 6 patients per dose level;dose level 1 (DL1) at 60 mg for 7 days, dose level 2 (DL2) 60 mg for 14 days, and dose level -1 (DL-1), 40 mg for 14 days, and enrollment was completed in May 2013.

The maximum tolerated dose was identified as 40 mg for 14 days or 60 mg for 7 days with the dose limiting toxicities being 1 case of each: Grade 3 hyponatremia, Grade 3 QT prolongation, Grade 3 pericardial effusion, and Grade 3 constrictive pericarditis. The most common (20%) treatment-related adverse events were nausea (42%), diarrhea (32%), anemia (26%), febrile neutropenia (26%), neutropenia (21%), fatigue (21%), pyrexia (21%) and thrombocytopenia (21%). The most common (10%) Grade 3 or 4 treatment-related adverse events were febrile neutropenia (26%), thrombocytopenia (21%), anemia (21%), neutropenia (21%), leucopenia (16%) and nausea (11%).

Complete responses were observed in 83% (15/18) patients evaluable for response, including 87.5% (7/8) of FLT3-ITD positive patients.

Ongoing Phase I Clinical Trial of Quizartinib as Maintenance Therapy Following an HSCT

In June 2012, we initiated a Phase 1 dose-escalating clinical trial to evaluate quizartinib as a maintenance therapy for patients with AML, irrespective of FLT3-ITD status, who have received an allogeneic HSCT and are currently in remission. We believe quizartinib dosed as a continuous maintenance therapy following an HSCT will increase duration of remission, thus increasing overall survival. The goal of this clinical trial will evaluate the safety and tolerability of quizartinib as a maintenance therapy with a goal of increasing the duration of remission and prolonging overall survival. We expect data from this trial to be presented at a future medical conference.

Ongoing Investigator-Sponsored Phase 1 Clinical Trial: AML18 Study

An ongoing investigator-sponsored Phase 1 dose-escalating clinical trial is evaluating quizartinib in combination with conventional chemotherapy in newly diagnosed AML patients over the age of 60, irrespective of FLT3-ITD status. Preliminary data from this study was presented at the 2013 Annual Meeting of ASH. The patients received quizartinib sequentially two days after each of two courses of ADE (Ara-C/ Daunorubicin/ Etoposide) followed by one course of daunorubicin, or DA. A total of 55 patients were enrolled in the study, of which 4 patients were FLT3-ITD positive, across four cohorts; 60 mg for 7 days, 60 mg for 14 days, 40 mg for 7 days, and 40 mg for 14 days. QT prolongation was the most frequent dose limiting toxicity, being observed in 3

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patients. The study demonstrated that quizartinib can be given sequentially after chemotherapy in older patients with newly diagnosed AML, and identified a maximum tolerated dose of 40 mg for 14 days. Complete responses were observed in 79% (n=33/42) patients evaluable for response, including 100% (n=4/4) of FLT3-ITD positive patients. A follow on investigator-sponsored Phase 3 clinical trial is planned.

Ongoing Investigator-Sponsored Phase 2/3 Clinical Trial: LI-1 Study

An ongoing investigator-sponsored Phase 2/3 clinical trial is evaluating quizartinib in combination with low dose cytarabine in previously untreated AML or myelodysplastic syndrome, or MDS, patients over the age of 60 who were unsuitable for standard induction chemotherapy. The patients are currently receiving 90mg of quizartinib daily for 21 days of each 28 day cycle. A total of 50 patients are planned to be enrolled prior to an analysis that will determine whether or not the clinical trial continues with additional patients.

Ongoing Investigator-Sponsored Phase1/2 Clinical Trial:

An ongoing investigator-sponsored Phase 1/2 dose escalation clinical trial is evaluating quizartinib in combination with either 5-azacitidine or low dose cytarabine for patients 18 years or older who had relapsed AML or MDS. The clinical trial is expected to proceed to Part 2 pending no safety concerns in Part 1 (dose escalation phase), where previously-untreated FLT3-ITD positive AML patients 60 years of age or older or FLT3-ITD positive AML patients 18 years of age or older in first relapse will be enrolled. Up to a total of 64 patients are planned to be treated on this clinical trial.

Investigator-Sponsored Phase 1 Clinical Trial: TACL Study

In addition to the clinical development plan for the treatment of adult patients, an investigator-sponsored Phase 1 clinical trial has been completed which evaluated quizartinib in pediatric patients with either relapsed acute lymphoblastic leukemia, or ALL, or relapsed AML. This clinical trial has been completed and data was presented at the 2013 Annual Meeting of ASH and is being sponsored by the Therapeutic Advances in Childhood Leukemia & Lymphoma Cooperative Group. A total of 22 patients were enrolled, of which 18 were evaluable for response and 20 were evaluable for toxicity. The maximum tolerated dose identified in this clinical trial was 60 mg per day. A follow-on Phase 2 clinical trial in pediatric patients is planned.

Companion Diagnostic

We have partnered with Genoptix in developing a validated companion diagnostic test to identify FLT3-ITD positive patients for inclusion in our clinical trials. Genoptix plans to prepare and submit a premarket approval application, or PMA, for this companion diagnostic to the FDA in connection with our NDA submission for quizartinib.

AC410: JAK2-Kinase Specific Inhibitor for Inflammatory Diseases

Our lead clinical-stage drug candidate that inhibits JAK2, AC410, is a potent, selective, orally-administered, small molecule inhibitor of JAK2, which has potential utility for the treatment of autoimmune and inflammatory diseases or as immunotherapy in oncology.

The JAK family comprises four intracellular, non-receptor tyrosine kinases: JAK1, JAK2, JAK3 and Tyk2. JAK plays a central role in the cytokine signaling processes within the immune system, and each family member mediates the signaling of a distinct, but overlapping, subset of cytokines. Inflammatory diseases are frequently characterized by an over-active immune response driven by pro-inflammatory cytokines. In recent years, JAK inhibitors have gained

significant attention as a mechanism of action in the treatment both of oncology and inflammatory diseases.

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Much of the clinical development of JAK inhibitors in autoimmune and inflammatory diseases has focused on JAK1 and JAK3, but given the complexity and heterogeneity of these diseases, we believe therapeutic opportunities exist for each of the individual JAK family members, and that there is no best JAK target. The cytokines believed to promote inflammation vary greatly across diseases, and it is unlikely that any one JAK target will prove most efficacious in all inflammatory diseases or patient subsets. Furthermore, we believe a selective approach to JAK inhibition has the potential to improve safety by narrowing the spectrum of activity, while maintaining efficacy by inhibiting the key cytokines driving inflammation.

Specifically, JAK2 mediates the signaling of a unique subset of cytokines that is distinct from JAK1 and JAK3. These cytokines include IL-6, IL-12, and IL-23 which play a key role in the autoimmune diseases such as psoriasis and rheumatoid arthritis, and IL-5, IL-13, and GM-CSF which play a key role in allergic diseases such as asthma. We believe this distinct activity could potentially deliver a competitive alternative to other JAK inhibitors while opening up first-in-class opportunities in novel therapeutic areas where JAK inhibitors have yet to be studied in the clinic.

Many of these same cytokines (especially IL-6 and IL-23) have been suggested to play an important role in the reprogramming of the tumor microenvironment into a pro-tumor state, resulting in a more aggressive cancer. A small molecule JAK2 inhibitor may provide a novel approach to immune therapy in oncology by repressing the pro-tumor microenvironment and enabling an anti-tumor response from the immune system.

Our initial JAK2 drug candidate, AC430, is a racemic mixture (50/50) of two enantiomers, AC410 and AC409, and was studied in a Phase 1 clinical trial. We have selected AC410 over AC430 and AC409 for further clinical development due to its superior pharmacokinetics as observed in this clinical trial. AC410 is an orally-administered small molecule drug candidate that inhibits JAK2 which can affect multiple pro-inflammatory cytokines. We believe it may provide additional therapeutic benefit in a wide range of autoimmune and inflammatory diseases when compared to current standards of care, including the convenience of once-daily oral dosing. We plan to advance AC410 to proof-of-concept clinical trials in one or more oncology, autoimmune and/or inflammatory diseases, independently or in collaboration with a strategic partner.

CSF1R Program: Selective Kinase Inhibitors for Oncology and Inflammation

Our lead compound in the program is a potent and exquisitely selective small molecule compound, AC708,that inhibits CSF1R.

Signaling through CSF1R controls the activation, proliferation and survival of macrophages, key mediators of immune system function.

The tumor microenvironment is increasingly understood to be a source of therapeutic targets for the treatment of cancer, and tumor associated macrophages, or TAMs, are thought to play a key role. TAMs, through cellular signaling, can promote angiogenesis (blood vessel formation in tumors), tumor survival and metastasis, and may confer resistance to current therapies. TAMs are dependent on CSF-1 (a cytokine that signals through CSF1R), and we believe that a drug that inhibits CSF1R could limit the influence of TAMs on the tumor microenvironment, and could be complementary and augment current cancer therapies. Over-activation of macrophages is believed to play a role in conditions such as rheumatoid arthritis, inflammatory bowel diseases, lupus nephritis, diabetic nephropathy, atherosclerosis, and even conditions such as obesity.

CSF1R is a member of the same kinase family as FLT3, cKIT, and PDGFR, and all share a highly similar structure. While the CSF1R kinase has been a target of interest in the pharmaceutical industry for several years, a key challenge has been the identification of inhibitors with sufficient selectivity for CSF1R (and minimal to no off-target activity on

FLT3, cKit and PDGFR) and there are currently no approved therapies that specifically target CSF1R. We believe that a CSF1R inhibitor that also provides potent inhibition of FLT3 and/or cKIT may result in myelosuppression, a side effect which would limit dosing in oncology indications and be unacceptable

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in non-oncology indications. We believe there is therapeutic potential for our CSF1R inhibitor program in oncology and/or autoimmune and inflammatory diseases due to maximized selectivity and potency on CSF1R and minimized/eliminated off-target activity.

We have discovered and are developing a potent compound, AC708, that is exquisitely selective for CSF1R, including significantly reduced activity against FLT3, cKIT and PDGFR. Characterization of this preclinical compound in *in vitro* and *in vivo* studies has demonstrated potent inhibition of CSF1R and inhibition of macrophage activity and proliferation. IND-enabling toxicology studies have shown AC708 to be generally well tolerated at exposures higher than what we believe will be necessary to achieve a therapeutic response in patients. We are expanding our pre-clinical studies of AC708 in oncology disease models to better define our plans for our Phase 1 and Phase 2 clinical trials. We plan to further develop this program independently or in collaboration with a strategic partner.

CEP-32496 BRAF Kinase Inhibitor for Oncology

CEP-32496 is a small molecule drug candidate we discovered that inhibits the BRAF kinase and is now being developed by Teva Pharmaceutical Industries Ltd., or Teva, pursuant to a collaboration agreement initiated in 2006 between Ambit and Cephalon, Inc. (subsequently acquired by Teva in October 2011). Pursuant to this collaboration, Teva has full responsibility for the worldwide development and commercialization of CEP-32496 and we are entitled to receive development, regulatory and commercialization milestones and sales-based royalty payments.

Our Strategic Alliances and Collaboration Agreements

Our Collaboration with Teva

In November 2006, we entered into an exclusive collaboration agreement with Cephalon, Inc., aimed at identifying and developing clinical candidates that demonstrate activity towards the two designated target kinases of the collaboration: the BRAF kinase and a second kinase determined by a joint research committee. In October 2011, Teva acquired Cephalon, Inc. Under the agreement, both parties contributed certain intellectual property to the collaboration and agreed to a period of exclusivity during which neither party would engage in any research related to a collaboration target compound with any third-party Teva is solely responsible for worldwide clinical development and commercialization of collaboration compounds, subject to our option, exercisable during certain periods following completion of the first proof-of-concept study in humans and only with the consent of Teva, to co-develop and co-promote CEP-32496.

Cephalon, Inc. paid us an upfront fee of $15.5 million as partial consideration for access to our profiling technology and the licenses we contributed to the collaboration. We have earned three milestone payments totaling $4.0 million under the agreement to date and we may be entitled to receive up to $44.5 million in additional payments upon the achievement of development, regulatory and sales milestones for CEP-32496, and up to $46.5 million in payments upon the achievement of development, regulatory and sales milestones for the second compound under the agreement. In addition, we may receive tiered royalty payments ranging from the mid-single digits to the low double digits calculated as a percentage of net sales of the collaboration compounds, including CEP-32496, subject to certain offsets. Royalties are payable to us on a product-by-product, country-by-country basis beginning on the date of the first commercial sale in a country and ending on the later of 10 years after the date of such sale in that country or the expiration date of the last to expire patent covering the licensed product in that country.

The collaboration portion of the agreement ended in November 2009, at which point we had completed all our research obligations under the agreement. The agreement remains in effect on a product-by-product, country-by-country basis until all royalty obligations expire. Both parties have a right to terminate the agreement early

if the other party enters bankruptcy or upon an uncured breach by the other party. Teva may also terminate the agreement in its discretion upon 90 days written notice to us, or if our available cash falls below a certain threshold.

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Our Collaboration with Genoptix

In September 2010, we entered into a collaboration agreement with Genoptix to develop a laboratory diagnostic test to identify patients that harbor ITD mutations in their FLT3 receptor tyrosine kinase. Under this agreement, Genoptix will contribute its expertise in developing laboratory tests and we will supply certain patient samples to the collaboration. Genoptix has the right to commercialize the approved test. We will initially pay for the development activities under the collaboration pursuant to an agreed-upon budget, and are entitled to single-digit tiered royalty payments from Genoptix until we have recouped the development costs plus an additional predetermined percentage of such costs. We intend for this test to be approved by the FDA as a companion diagnostic test in concert with quizartinib.

We and Genoptix may assign this agreement to a third party in connection with the transfer or sale of all or substantially all of the business to which the agreement relates, whether by merger, sale of stock, sale of assets or otherwise, provided that in the event of such a transaction with a third party, intellectual property rights of such third-party will not be included in the intellectual property rights licensed under our agreement with Genoptix to the extent such intellectual property rights would not have been licensed under the agreement in the absence of such transaction.

Our agreement with Genoptix expires when the last payment obligation of either party under the agreement is fulfilled. Both parties have a right to terminate the agreement early upon an uncured material breach by the other party. Genoptix may terminate the agreement upon 45 days notice for an unresolved dispute between the parties regarding the development of the laboratory diagnostic test, upon 30 days notice if there is an unresolved dispute regarding our payment of specified development costs and upon written notice if we, our affiliates, or our sublicensees of certain intellectual property, where we do not, within ten days of receipt of notice from Genoptix, terminate such sublicense, contest or assist other parties in contesting Genoptix s rights regarding such intellectual property. We may terminate the agreement upon 60 days notice for any reason subject to our payment of any outstanding development costs, and immediately if Genoptix or a party providing services to Genoptix relating to the development of the laboratory diagnostic test is debarred under the provisions of the Generic Drug Enforcement Act of 1992.

Intellectual Property

We are building an intellectual property portfolio around our clinical drug programs and our drug discovery programs. An essential part of our strategy for portfolio building is to seek patent protection in the United States and in major market countries that we consider important to the development of our business worldwide.

In addition to patent protection, we seek to rely on trade secret protection, trademark protection and know-how to expand our proprietary position around our chemistry, biology, as well as other technologies and other discoveries and inventions that we consider important to our business. We also seek to protect our intellectual property in part by entering into confidentiality agreements with third parties with whom we share proprietary or confidential information in the course of business discussions, and by having confidentiality terms in our agreements with our employees, consultants, scientific advisors, clinical investigators and other contractors and also by requiring our employees, commercial contractors, and certain consultants and investigators, to enter into invention assignment agreements that grant us ownership of any discoveries or inventions made by them in the course of working with us.

Our success depends in part on our ability to obtain and maintain proprietary protection for our drug candidates and other discoveries, inventions, trade secrets and know-how that are critical to our business operations. Our success also depends in part on our ability to operate without infringing the proprietary rights of others, and in part, on our ability to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual

property is provided under Risk Factors under the subsection Risks Related to Our Intellectual Property .

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Quizartinib

We have 43 issued US and foreign composition of matter patents that provide protection for the quizartinib molecule, both generically and specifically. Our key US composition of matter patent, US 7,820,657, will expire in 2028 if we pay all maintenance fees when due and if unchallenged prior to expiration, with the possibility of additional term from patent term extension that may be granted under the relevant statutes upon our application for such extension. Corresponding foreign composition of matter patents have issued in Australia, China, Hong Kong, Japan, Malaysia, Mexico, New Zealand, Russia, Singapore and South Africa and 31 European countries, which provide patent protection through 2027, absent any available patent term extensions.

In addition to the composition of matter patent, we have issued US patents that include dosing method patents that cover dosing regimens for monotherapy and combination therapies for quizartinib, method of treatment patents that cover the use of quizartinib for hematological cancers and a metabolite patent that the covers the active metabolite of quizartinib. We also have pending US applications that cover the stable crystalline forms of quizartinib, quizartinib formulations and methods for manufacturing the quizartinib molecule. Corresponding foreign cases have either issued as patents or are currently pending as patent applications. These additional patents will provide further protection with later expirations ranging from 2028-2030, and possibly to 2033 if the latest filed patent applications are to issue, absent any available patent term extensions.

AC430/AC410

We have an issued US composition of matter patent that provides generic protection for the molecule AC430 and its enantiomer AC410 and that also provides specific protection for AC430. This patent, US 8,349,851, will expire in 2030 if we pay all maintenance fees when due, and if it is not challenged prior to expiration, with the possibility of additional term from patent term extension that may be granted under the relevant statutes upon our application for such extension. We also have a second issued US composition of matter patent that covers the stable crystalline forms of AC430 having an expiration of 2031, absent any available patent term extension. Corresponding foreign patent applications are currently pending. We are pursuing specific protection of the enantiomer AC410, in patent applications filed in the US, Europe, Canada, Australia and Japan. We intend to file additional U.S. and foreign applications directed, for example, to formulations, therapeutic uses, combination therapies and methods of manufacture, and other inventions to the extent such inventions or discoveries are made.

AC708

We have filed patent applications that provide both generic and specific composition of matter protection for our CSF1R candidate AC708, under the PCT and also in the U.S., Argentina and Taiwan. We intend to build the patent portfolio around this candidate, and intend to file additional U.S. and foreign patent applications to the extent inventions and discoveries are made in our ongoing program around CSF1R.

Trademark

We seek trademark protection in the United States and internationally where available and when we deem appropriate. We have obtained registrations for the AMBIT trademark, which we use in connection with our pharmaceutical research and development services as well as our clinical-stage products. We currently have such registrations for AMBIT in the United States, Europe and Japan.

Sales and Marketing

We currently do not have a commercial organization for the marketing, sales and distribution of pharmaceutical products. We intend to build the commercial infrastructure necessary to effectively support the commercialization of quizartinib and future drug candidates, if approved, in North America and to partner with third parties for commercialization in other markets.

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The commercial infrastructure of specialty oncology products typically consists of a targeted, specialty sales force that calls on a limited and focused group of physicians supported by sales management, internal sales support, an internal marketing group and distribution support. Additional capabilities important to the oncology marketplace include the management of key accounts such a managed care organizations, group-purchasing organizations, specialty pharmacies, oncology group networks, and government accounts. Based on the number of physicians that treat AML and the size of competitive sales forces, we believe that we can effectively target the relevant audience for quizartinib in North America by establishing a sales force either internally or through a contract sales force. To develop the appropriate commercial infrastructure, we will have to invest significant amounts of financial and management resources, some of which will be committed prior to any confirmation that quizartinib will be approved.

Manufacturing

We do not own or operate manufacturing facilities for the production of quizartinib or other drug candidates that we develop, nor do we have plans to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials, active pharmaceutical ingredient and finished products for our preclinical research and clinical trials. We do not have any current contractual arrangements for the manufacture of commercial supplies of quizartinib or any other drug candidates that we develop. Prior to receipt of approval from the FDA, we intend to enter into agreements with third-party contract manufacturers for the commercial production of quizartinib. We currently employ internal resources and third-party consultants to manage our manufacturing contractors.

Competition

A number of multinational pharmaceutical companies, as well as large biotechnology companies, including Abbvie Inc., Akinion Pharmaceuticals AB, Amgen Inc., ARIAD Pharmaceuticals, Inc., AROG Pharmaceuticals, LLC, ArQule, Inc., Astellas, AstraZeneca plc, Bayer AG, Celgene Corporation, Daiichi-Sankyo Company Limited, Galapagos NV, GlaxoSmithKline plc, Incyte Corporation, Janssen Pharmaceuticals, Inc., Johnson & Johnson, Eli Lilly and Company, Novartis, Onyx Pharmaceuticals, Inc., Pfizer, Rigel Pharmaceuticals, Inc., F. Hoffman-LaRoche Ltd, or Roche, and Vertex Pharmaceuticals Incorporated, are pursuing the development or are currently marketing pharmaceuticals that target the kinases or kinase-signaling pathways and in the specific therapeutic areas on which we are focusing. It is probable that the number of companies seeking to develop products and therapies for the treatment of unmet needs in oncology, autoimmune and inflammatory diseases will increase.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of drug candidates, obtaining FDA and other regulatory approvals of drug candidates and the commercialization of those products. Accordingly, our competitors may be more successful than we may be in obtaining approval for drugs and achieving widespread market acceptance. Our competitors drugs may be more effective, or more effectively marketed and sold, than any drug we may commercialize and may render our drug candidates obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our drug candidates. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available.

Competition for Quizartinib

Pfizer s Sutent® (sunitinib) and Bayer s and Onyx s Nexavar® (sorafenib), two multi-kinase inhibitors that inhibit the FLT3 kinase, are approved for the treatment of certain solid tumors; however, these drugs also inhibit other kinases with equal or greater potency and are not approved for the treatment of AML. Sutent ® is approved as monotherapy

for renal cell carcinoma, or RCC, for gastrointestinal stromal tumors, or GIST, and pancreatic neuroendocrine tumors, or pNET; and Nexavar ® is approved as monotherapy for advanced RCC and unresectable hepatocellular cancer, or HCC. Each of these drugs is believed to work through inhibition of kinases

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other than FLT3. Because of the lack of treatment options for this patient population, commercially-available kinase inhibitors, such as sunitinib and sorafenib, are often used off-label for the treatment of AML despite the low response rate observed with these drugs. Currently there are no approved therapies for relapsed/refractory AML beyond traditional chemotherapy. We are aware of only one FLT3 inhibitor, Novartis PKC-412 (midostaurin), that is in a Phase 3 clinical trial for the treatment of newly diagnosed FLT3-ITD positive AML patients. Additionally, FLT3 inhibitors in Phase 1 and Phase 2 development for AML include AROG Pharmaceuticals crenolanib, ARIAD Pharmaceuticals Iclusig® (ponatinib), Daiichi-Sankyo s PLX-3397 and Akinion Pharmaceuticals AKN-028.

Competition for AC410

Pfizer s Xeljanz® (tofacitinib), a pan-JAK inhibitor, was recently approved in the United States for the treatment of RA, becoming the first inhibitor of the JAK family of kinases to be approved worldwide to treat an inflammatory disease. There are several companies with inhibitors of the JAK family of kinases in clinical development for inflammatory disease, including Astellas/Janssen Pharmaceuticals, Incyte/Eli Lilly, Galapagos/Abbvie, Rigel Pharmaceuticals/AstraZeneca and Vertex, and, to our knowledge, there are no clinical-stage JAK inhibitors targeting respiratory diseases.

Competition for CSF1R

We are not aware of any commercialized products that target CSF1R. There are a number of companies with oral small molecule CSF1R inhibitors in clinical development, including Celgene/Array, Daiichi-Sankyo and Johnson & Johnson. In addition, we are aware of several companies with biologic CSF1R inhibitors in clinical development, including Amgen, Eli Lilly, Pfizer and Roche.

Competition for CEP-32496

Daiichi-Sankyo s and Roche s Zelboraf® (vemurafenib), a BRAF kinase inhibitor, was approved by the FDA in 2011 for the treatment of metastatic melanoma patients harboring the V600E BRAF mutation, becoming the first BRAF kinase inhibitor to be approved worldwide. We are aware of a number of companies with BRAF inhibitors in clinical development, including ArQule, GlaxoSmithKline and Novartis.

Government Regulation and Product Approval

Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug products and companion diagnostic devices such as those we and our partners are developing. Quizartinib and any other drug candidates that we develop must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agency before they may be legally marketed in foreign countries. In addition, the FDA is currently requiring regulatory approval of a companion diagnostic for market approval of quizartinib.

United States Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act, or FDCA, and implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the

applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include, among other actions, refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls or withdrawals from the market, product seizures, total

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or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us. The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- Completion of extensive preclinical laboratory tests, preclinical animal studies and formulation studies in accordance with applicable regulations, including the FDA s Good Laboratory Practice, or GLP, or other applicable regulations;
- Submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- Performance of adequate and well-controlled human clinical trials in accordance with applicable regulations, including the FDA s current good clinical practices, or GCPs, to establish the safety and efficacy of the proposed drug for its proposed indication;
- Submission to the FDA of an NDA for a new drug;
- A determination by the FDA within 60 days of its receipt of an NDA to file the NDA for review;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the drug is produced to assess compliance with the FDA s current good manufacturing practice standards, or cGMP, to assure that the facilities, methods and controls are adequate to preserve the drug s identity, strength, quality and purity;
- Potential FDA audit of the preclinical and/or clinical trial sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Before testing any compounds with potential therapeutic value in humans, the drug candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the drug candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human studies. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the proposed clinical trials and places the IND on clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any

outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a drug candidate at any time before or during clinical trials due to safety concerns or non-compliance. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trial.

Each new clinical trial protocol, and any subsequent amendments to the protocol, must be submitted to the FDA for review and to an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted, for approval. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

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Human 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 excretion, the side effects associated with increasing doses, and if possible, to gain early evidence of effectiveness. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

Phase 2. The drug is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases or conditions and to determine dosage tolerance, optimal dosage and dosing schedule.

Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall benefit/risk ratio of the product and provide an adequate basis for product approval. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA. Phase 3 clinical trials usually involve several hundred to several thousand participants.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, FDA may mandate the performance of Phase 4 studies.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA, the IRB, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, 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. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access to certain data from the study. We may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final 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 shelf life.

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The application includes both negative or ambiguous results of preclinical and clinical trials as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval,

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the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA. The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances.

In addition, under the Pediatric Research Equity Act, or PREA, an NDA or supplement to an NDA must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s).

The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has 12 months from its date of receipt in which to complete its initial review of a standard NDA for a new molecular entity and respond to the applicant, and eight months from date of receipt for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs, and the review process is often significantly extended by FDA requests for additional information or clarification.

After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product s identity, strength, quality and purity. The FDA may refer applications for novel drug or biological products or drug or biological products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with GCP requirements. After the FDA evaluates the application, manufacturing process and manufacturing facilities, 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 usually describes all of the specific deficiencies in the NDA identified by the FDA. The Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further,

the FDA may require that certain contraindications, warnings or precautions be included in the product labeling or may condition the approval of the NDA on other changes to the proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post-market studies or clinical trials. For

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example, the FDA may require Phase 4 testing which involves clinical trials designed to further assess a drug safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. Regulatory approval of oncology products often requires that patients in clinical trials be followed for long periods to determine the overall survival benefit of the drug. The FDA may also determine that a risk evaluation and mitigation strategy, or REMS, is necessary to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the NDA without an approved REMS, if required. A REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States there is no reasonable expectation that the cost of developing and making a drug product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product designation must be requested before submitting an NDA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. The designation of such drug also entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug or biological product as defined by the FDA or if our drug candidate is determined to be contained within the competitor s product for the same indication or disease. If a drug product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar but not identical benefits in that jurisdiction.

We currently have Orphan Drug Designation for quizartinib for the treatment of AML in the United States and the European Union.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drug products that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. Unique to a Fast Track product, the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

We received Fast Track designation for quizartinib for treatment of patients 60 years of age or older with FLT3-ITD positive AML in first relapse or refractory to first line chemotherapy and treatment of patients 18 years or older

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with FLT3-ITD positive AML in second relapse or refractory to second line salvage therapy. Even though we received Fast Track designation for quizartinib, the FDA may later decide that quizartinib no longer meets the conditions for qualification. In addition, Fast Track designation may not provide us with a material commercial advantage.

Any product, submitted to the FDA for approval, including a product with a Fast Track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. The FDA currently accepts CR as a surrogate endpoint for the basis of accelerated review of products for the treatment of AML. Fast Track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

Companion Diagnostic Review and Approval

In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Diagnostic tests are classified as medical devices under the FDCA. Unless an exemption applies, diagnostic tests require marketing clearance or approval from the FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and premarket approval, or PMA approval.

Our drug candidate quizartinib currently relies upon the conduct of a companion diagnostic test to select patients with the FLT3-ITD mutation. Presently, the FLT3-ITD mutation companion diagnostic test is available only as a Laboratory Developed Test, or LDT, that is commercialized by laboratories certified under the Clinical Laboratory Improvement Amendments of 1988, or CLIA. LDTs are diagnostic tests developed and performed by a single laboratory. The FDA currently exercises enforcement discretion and does not enforce the requirements applicable to medical devices against LDTs, but has indicated its intent to end its enforcement discretion and begin enforcing its medical device regulations on such tests in a risk-based manner. Approval of our quizartinib drug candidate, however, will require FDA approval of a premarket approval application, or PMA, for a reproducible, validated diagnostic test to be used with quizartinib.

The PMA process is costly, lengthy, and uncertain, although the PMA review for a FLT3-ITD mutation test is currently planned to occur concurrently with the development and review of an NDA for quizartinib. PMA applications must be supported by valid scientific evidence, which typically requires extensive data, including technical, preclinical, clinical and manufacturing data, to demonstrate to the FDA s satisfaction the safety and effectiveness of the device. For diagnostic tests, a PMA application typically includes data regarding analytical and clinical validation studies. As part of its review of the PMA, the FDA will conduct a pre-approval inspection of the manufacturing facility or facilities to ensure compliance with the Quality System Regulation, or QSR, which requires manufacturers to follow design, testing, control, documentation and other quality assurance procedures. FDA review

of an initial PMA application is required by statute to take between six to ten months, although the process typically takes longer, and may require several years to complete. The receipt and timing of PMA approval may have a significant effect on the receipt and timing of commercial approval for quizartinib. If the FDA evaluations of both the PMA application and the manufacturing facilities are favorable, the FDA will either issue an approval letter or an approvable

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letter, which usually contains a number of conditions that must be met in order to secure the final approval of the PMA. If the FDA s evaluation of the PMA or manufacturing facilities is not favorable, the FDA will deny approval of the PMA or issue a not approvable letter. A not approvable letter will outline the deficiencies in the application, and where practical, will identify what is necessary to make the PMA approvable. The FDA may also determine that additional clinical trials are necessary, in which case the PMA approval may be delayed for several months or years while the trials are conducted and then the data submitted in an amendment to the PMA. Once granted, PMA approval may be withdrawn by the FDA if compliance with post approval requirements, conditions of approval or other regulatory standards is not maintained or problems are identified following initial marketing.

In July 2011, the FDA issued a draft guidance document addressing the development and approval process for In Vitro Companion Diagnostic Devices. According to the draft guidance, for novel therapeutic products such as quizartinib, the PMA for a companion diagnostic device should be developed and approved or cleared contemporaneously with the therapeutic. While this draft guidance is not yet finalized, we believe our program for the development of our companion diagnostic is consistent with the draft guidance as proposed.

Medical devices, including companion diagnostics, are subject to pervasive and ongoing regulatory obligations, including the submission of medical device reports, continued adherence to the Quality Systems Regulation, recordkeeping and product labeling, as enforced by the FDA and comparable state authorities.

Post-Approval Requirements

Any drug products for which we receive FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the drug s approved labeling (known as off-label use), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses.

In addition, quality control and manufacturing procedures must continue to conform to applicable manufacturing requirements after approval to ensure the long term stability of the drug product. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved NDA, including, among other things, recall or withdrawal of the product from the market. In addition, changes to the manufacturing process are strictly regulated, and depending on the significance of the change, may require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, risk minimization action plans and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the

distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, warning letters from the FDA, mandated corrective advertising

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or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product s approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA s policies may change, which could delay or prevent regulatory approval of our products under development.

Additional U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services (formerly the Health Care Financing Administration), other divisions of the United States Department of Health and Human Services (e.g., the Office of Inspector General), the United States Department of Justice and individual United States Attorney offices within the Department of Justice, and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the anti-fraud and abuse provisions of the Social Security Act, the False Claims Act, the privacy provisions of the Health Insurance Portability and Accountability Act, or HIPAA, and similar state laws, each as amended. Pricing and rebate programs must comply with the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990 and the Veterans Health Care Act of 1992, each as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Under the Veterans Health Care Act, or VHCA, drug companies are required to offer certain drugs at a reduced price to a number of federal agencies including United States Department of Veterans Affairs and United States Department of Defense, the Public Health Service and certain private Public Health Service designated entities in order to participate in other federal funding programs including Medicare and Medicaid. Recent legislative changes purport to require that discounted prices be offered for certain United States Department of Defense purchases for its TRICARE program via a rebate system. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulations.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our drug candidates, some of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product s approval date. The patent term restoration period is

generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the

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approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA.

Market exclusivity provisions under the FDCA can also delay the submission or the approval of certain marketing applications. 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. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, 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-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 or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness. Orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity, except in certain circumstances. Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued Written Request for such a trial.

Europe / Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products.

Whether or not we or our collaborators obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a clinical trial application, or CTA, must be submitted to each country s national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country s requirements, clinical trial development may proceed.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the NDA or BLA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements.

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For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we or our collaborators fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug candidates for which we or our collaborators obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we or our collaborators receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We or our collaborators may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our drug candidates may not be considered medically necessary or cost-effective. A payor s decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future legislation could limit payments for pharmaceuticals such as the drug candidates that we are developing.

Different pricing and reimbursement schemes exist in other countries. In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular drug candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any drug candidates for which we or our collaborators receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement

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rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Employees

As of December 31, 2013, we employed 53 employees, 49 of whom are full-time, 12 of whom hold Ph.D. or M.D. degrees, 33 of whom were engaged in research and development activities and 20 of whom were engaged in business development, finance, information systems, facilities, human resources or administrative support. None of our employees is subject to a collective bargaining agreement. We consider our relationship with our employees to be good.

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Item 1A. Risk Factors.

You should carefully consider the following risk factors, as well as the other information in this report, before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to Our Business and Industry

We are highly dependent on the success of our lead drug candidate, quizartinib, which is still in clinical development, and we cannot give any assurance that it, or any other drug candidates, will receive regulatory approval, which is necessary before they can be commercialized.

Our future success is substantially dependent on our ability to obtain regulatory approval for, and then successfully commercialize our lead drug candidate, quizartinib. Our other drug candidates are in earlier stages of development. Our business depends entirely on the successful development and commercialization of our drug candidates. We have not completed the development of any drug candidates; we currently generate no revenues from sales of any drugs, and we may never be able to develop a marketable drug.

Quizartinib will require additional clinical development, evaluation of clinical, preclinical and manufacturing activities, regulatory approval in multiple jurisdictions, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenues from product sales. The U.S. Food and Drug Administration, or FDA, has also informed us that an approved companion diagnostic is required in order to obtain approval of quizartinib. Companion diagnostics are subject to regulation as medical devices and must be separately approved for marketing by the FDA. We are not permitted to market or promote quizartinib, or any other drug candidates before we receive regulatory approval from the FDA and comparable foreign regulatory authorities, and we may never receive such regulatory approvals.

We plan to initiate a randomized, comparative Phase 3 clinical trial of quizartinib in patients with relapsed/refractory acute myeloid leukemia, or AML, in the second quarter of 2014. There is no guarantee that this trial will commence or be completed on time or at all. Prior to receiving approval to commercialize quizartinib or future product candidates, if any, in the United States or internationally, we must demonstrate with substantial evidence from well-controlled clinical trials, to the satisfaction of the FDA and other regulatory authorities, that such product candidates are safe and effective for their intended uses. The results from preclinical studies and clinical trials can be interpreted in different ways, and the favorable results from previous trials of quizartinib may not be experienced in the Phase 3 clinical trial. Even if we believe the preclinical or clinical data are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. We maintain frequent, ongoing dialog with the FDA and other regulatory bodies regarding our clinical trial design, including the patient selection criteria, dosing plan and statistical analysis plan. There is a risk that the FDA or other regulatory agencies could at any time raise objections. Any such objections could delay the initiation or completion of our Phase 3 clinical trial. Although we believe that our discussions with the FDA support the potential approval of quizartinib for the treatment of patients with relapsed/refractory AML who harbor the FLT3-ITD mutation, which we refer to as FLT3-ITD positive, based on positive results from the Phase 3 clinical trial without the need to conduct additional clinical trials, the FDA has substantial discretion in the approval process and may not grant approval based on data from this trial.

Even if the trial is successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do. If the results of the trial are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant additional resources to conduct

additional trials in support of potential approval of quizartinib.

We cannot anticipate when or if we will seek regulatory review of quizartinib for any other indications. We have not previously submitted a New Drug Application, or NDA, to the FDA, or similar drug approval filings to

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comparable foreign authorities, or received marketing approval for any drug candidate, and we cannot be certain that quizartinib will be successful in clinical trials or receive regulatory approval for any indication. If we do not receive regulatory approvals for and successfully commercialize quizartinib on a timely basis or at all, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market quizartinib, our revenues will be dependent, in part, on our collaborator s ability to obtain regulatory approval of the companion diagnostic to be used with quizartinib, our collaborator s ability to commercialize the test as well as the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for the treatment of AML are not as significant as we estimate, our business and prospects will be harmed.

We plan to seek regulatory approval to commercialize quizartinib both in the United States and in select foreign countries. While the scope of regulatory approval is similar in other countries, in some countries there are additional regulatory risks and we cannot predict success in these jurisdictions.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies and trials may not be predictive of future trial results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our drug candidates may not be predictive of the results of later-stage clinical trials. Drug candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or safety profiles, notwithstanding promising results in earlier trials.

We may experience delays in clinical trials of our drug candidates. We plan to initiate a Phase 3 clinical trial of quizartinib in the second quarter of 2014 in patients with relapsed/refractory AML who harbor the FLT3-ITD mutation. We may encounter delays if we are unable to commence the Phase 3 clinical trial on time or enroll enough patients to complete the Phase 3 clinical trial or other clinical trials of quizartinib. Patient enrollment depends on many factors, including the size of the patient population, the nature of the protocol, the proximity of patients to clinical sites, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same indication, and the eligibility criteria for the trial. Patients participating in our trials may elect to leave our trials and switch to alternative treatments that are available to them, either commercially or on an expanded access basis, or in other clinical trials. Competing treatments include nucleoside analogs, anthracyclines and hypomethylating agents. Moreover, when one product candidate is evaluated in multiple clinical trials simultaneously, patient enrollment in ongoing trials can be adversely affected by negative results from completed trials. In the Phase 3 clinical trial, we plan to enroll patients with relapsed/refractory AML, who are FLT3-ITD positive, which can be a difficult patient population to recruit.

We are currently evaluating quizartinib in other indications in AML and plan, in the future, to initiate additional clinical trials in AML and other indications. We do not know whether ongoing clinical trials will be completed on schedule or at all, or whether planned clinical trials, including our Phase 3 clinical trial, will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

- obtaining regulatory approval to commence a trial;

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

obtaining institutional review board approval at each clinical trial site;

recruiting suitable patients to participate in a trial;

developing and validating the companion diagnostic to be used in the trial on a timely basis;

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having patients complete a trial or return for post-treatment follow-up;

clinical trial sites deviating from trial protocol or dropping out of a trial;

adding new clinical trial sites; or

manufacturing sufficient quantities of drug candidates for use in clinical trials.

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, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians and patients perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and, while we have agreements governing their committed activities, we have limited influence over their actual performance.

We could encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our drug candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, our collaborators, the institutional review boards, or IRBs, in the institutions in which such trials are being conducted, the Data Safety Monitoring Board, or DSMB, for such trial, or by the FDA or other regulatory authorities 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 candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of quizartinib or any of our other 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 product development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly. Furthermore, 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.

The FDA regulatory approval process is lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for quizartinib or our other drug candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and similar foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials, depending upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a drug candidate s clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any drug candidate.

Quizartinib and our other drug candidates could fail to receive regulatory approval for many reasons, including the following:

the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;

we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a drug candidate is safe and effective for its proposed indication;

the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;

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- we may be unable to demonstrate that a drug candidate s clinical and other benefits outweigh its safety risks;

- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;

- the data collected from clinical trials of our drug candidates may not be sufficient to the satisfaction of FDA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or foreign jurisdictions, on an accelerated basis or otherwise;

- the FDA or comparable foreign regulatory authorities may not accept new surrogate endpoints, which are endpoints intended to substitute for clinical endpoints, as a basis for submission of an NDA or other comparable submission in foreign jurisdictions or as a basis for regulatory approval on an accelerated basis or otherwise;

- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we or our collaborators contract for clinical and commercial supplies;

- the FDA or comparable foreign regulatory authorities, as applicable, may fail to approve the premarket approval application, or PMA, for the companion diagnostic we are developing with Genoptix Medical Laboratory, a Novartis company, or Genoptix, or may require approval of other diagnostic tests; and

- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failure to obtain regulatory approval to market quizartinib, or any of our other drug candidates, which would significantly harm our business, prospects, financial condition and results of operations. In addition, even if we were to obtain approval, regulatory authorities may approve any of our drug candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a drug candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that drug candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our drug candidates.

We will need to raise additional funding, which may not be available on acceptable terms, or at all.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to:

continue the clinical development of quizartinib in AML, including our clinical trials and our planned Phase 3 clinical trial, as well as the preclinical and clinical development of other drug candidates;

prepare regulatory submissions for regulatory approval of quizartinib;

continue our research and development programs to advance our internal product pipeline; and

launch and commercialize quizartinib and any other drug candidates for which we receive regulatory approval, including building our own commercial capabilities to sell, market, and distribute quizartinib in North America, if approved.

Our future funding requirements and sources will depend on many factors, including, the rate of progress and cost of our clinical trials, the need for additional patients in any of our clinical trials, and the need for additional clinical trials or data analyses. We will require additional capital for the further development and commercialization of quizartinib and our other drug candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

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We cannot be certain that additional funding will be available on acceptable terms, or at all. If we raise any debt financing, the terms of such debt could restrict our operating and financial flexibility. If we are unable to raise additional capital or debt financing in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our drug candidates or other research and development initiatives. We also could be required to:

- seek collaborators for quizartinib or one or more of our other current or future drug candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available;
- relinquish or license on unfavorable terms our rights to drug candidates that we otherwise would seek to develop or commercialize ourselves; or
- license or acquire additional drug candidates.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

We currently rely on Genoptix to develop the companion diagnostic test for quizartinib and in the future will rely on a third party to obtain marketing approval of such test, which will be required to market quizartinib in the United States. There is no guarantee that the FDA will grant timely approval of this test, if at all, and failure to obtain such timely approval would adversely affect our ability to obtain approval for quizartinib.

We intend to initially seek approval of quizartinib in relapsed/refractory FLT3-ITD positive AML patients. The initial proposed drug label being sought for quizartinib specific to this patient population would indicate a potential for enhanced efficacy and/or a greater likelihood of a positive response in patients that carry the FLT3-ITD positive genotype. Accordingly, the Phase 3 clinical trial uses a diagnostic test to select patients that are FLT3-ITD positive. In the United States, the FDA requires that the diagnostic test used to select patients in a pivotal trial be approved in parallel with the drug candidate as a companion diagnostic. A companion diagnostic is an in vitro diagnostic device that provides information that is essential for the safe and effective use of a corresponding therapeutic product. We believe a companion diagnostic to test for the FLT3-ITD positive genotype will be required for the approval of quizartinib. Companion diagnostics for any other genetic testing used to identify a target population for quizartinib that are determined to be necessary for its safety and efficacy will also be required. Companion diagnostics are subject to regulation as medical devices by the FDA and may be subject to regulation by comparable regulatory authorities in various foreign countries. The process of complying with the requirements of the FDA and comparable foreign agencies to support marketing authorization of a companion diagnostic is costly, time consuming and burdensome.

We do not develop companion diagnostics internally and thus we are dependent on the sustained cooperation and effort of third parties in developing and obtaining approval for these companion diagnostics. We have entered into an agreement with Genoptix, pursuant to which Genoptix will be responsible for developing the companion diagnostic and obtaining marketing authorization from the FDA. We believe Genoptix will need to submit a premarket approval application, or PMA, for such test, which we anticipate will happen in parallel with our submission of an NDA for quizartinib in accordance with FDA guidance that a novel therapeutic product and companion diagnostic device should generally be developed and approved contemporaneously to support the therapeutic product s safe and effective use. We currently do not believe that any clinical trials other than the quizartinib Phase 3 clinical trial will be required

to support the PMA for the companion diagnostic. However, the FDA may require Genoptix to perform further tests requiring access to patient samples for the test submission and/or future products. We intend to provide access to patient samples to Genoptix for such purposes and our informed consents with patients allow us to permit a third party to test these samples, as required.

We and Genoptix may encounter difficulties in developing and obtaining approval for the companion diagnostic, including issues relating to the selectivity/specificity, analytical validation, reproducibility, or clinical validation of the device. Despite the time and expense expended, regulatory approval of a companion diagnostic

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is never guaranteed. Any delay or failure by Genoptix to develop or obtain regulatory approval of the companion diagnostic could delay or prevent approval of quizartinib. In addition, while Genoptix has the right under our collaboration agreement to commercialize the companion diagnostic, it is not obligated to do so. Genoptix may elect to not commercialize, or even if it does elect to commercialize the companion diagnostic, Genoptix may decide to discontinue selling or manufacturing the companion diagnostic. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternate diagnostic test for use in connection with the development and commercialization of quizartinib or do so on commercially acceptable terms, which could adversely affect and/or delay the development or commercialization of quizartinib. In addition, Genoptix or any other diagnostic company may encounter production difficulties that could constrain the supply of the companion diagnostic, and both Genoptix and we may have difficulties gaining acceptance of the use of the companion diagnostic in the clinical community. If such companion diagnostic fails to gain market acceptance, it would have an adverse effect on our ability to derive revenues from sales of quizartinib. The commercial launch of quizartinib may be significantly and adversely affected if Genoptix is unable to obtain FDA approval of the companion diagnostic test in parallel with the approval of quizartinib or at all, or if a third party is unable to commercialize the test successfully and in a manner that effectively supports our commercial efforts.

Adverse side effects or other safety risks associated with our drug candidates could delay or preclude approval of quizartinib or any of our other current or future drug candidates, cause us to suspend or discontinue clinical trials, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our drug candidates could result in the delay, suspension or termination of our clinical trials by us, our collaborators, IRBs, the FDA or other regulatory authorities for a number of reasons. If we elect or are required to delay, suspend or terminate any clinical trial of any drug candidates that we develop, the commercial prospects of such drug candidates will be harmed and our ability to generate product revenues from any of these drug candidates will be delayed or eliminated. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

To date, the clinical development program for quizartinib includes over 480 patients treated in our Phase 1 and Phase 2 clinical trials in relapsed/refractory AML. The adverse events we have observed to date are manageable and the most common all grade treatment-emergent adverse events (reported in ³ 20% of subjects) in our Phase 2 clinical trials included gastrointestinal toxicities, fatigue, anemia, QT prolongation (changes in the patient s electrocardiogram pattern), and dysgeusia (distortion of the sense of taste). Overall, there were no major differences between safety findings in FLT3-ITD positive and FLT3-ITD negative patients or between the Phase 1 and Phase 2 clinical trials. QT prolongation is a common adverse event associated with multiple other kinase inhibitors and may be a class effect. The majority of cases of QT prolongation with quizartinib are asymptomatic and occur within the first month of treatment. Additionally, the majority of patients that experienced QT prolongation did not discontinue quizartinib treatment due to this adverse event. Nonetheless, QT prolongation may be associated with changes in electric conduction in the heart and may cause irregularities of the heart beat which could be potentially serious, life-threatening or fatal and require ECG monitoring and treatment. To date, there has been one case of Grade 4 QT interval prolongation with Torsade de pointes (an abnormal cardiac rhythm) in a patient taking quizartinib with multiple concomitant medications in our Phase 2 clinical trial. Results of our current and anticipated trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our drug candidates for any or all targeted indications. In addition, the drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

Additionally if quizartinib or any of our other drug candidates receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh its risks, which in the case of quizartinib may include, among other things, a medication guide outlining the risks of QT

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prolongation for distribution to patients and a communication plan to health care practitioners. We could also be required to include a black box warning on the label. Furthermore, if we or others later identify undesirable side effects caused by the product, 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 label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way quizartinib is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of quizartinib or the particular drug candidate at issue and could significantly harm our business, prospects, financial condition and results of operations.

If we are unable to obtain FDA approval of our drug candidates, we will not be able to commercialize them in the United States and our business will be adversely impacted.

We need FDA approval prior to marketing our drug candidates in the United States, and in the case of quizartinib, we must also ensure approval of a companion diagnostic. If we fail to obtain FDA approval to market our drug candidates, we will be unable to sell our drug candidates in the United States, which will significantly impair our ability to generate any revenues.

This regulatory review and approval process, which includes evaluation of preclinical studies and clinical trials of our drug candidates as well as the evaluation of our manufacturing processes and our third-party contract manufacturers facilities, is lengthy, expensive and uncertain. To receive approval, we must, among other things, demonstrate with substantial evidence from clinical trials that the drug candidate is both safe and effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the approval requirements typically takes several years and the time needed to satisfy them may vary substantially, based on the type, complexity and novelty of the pharmaceutical product. We cannot predict if or when we might receive regulatory approvals for any of our drug candidates currently under development. Moreover, any approvals that we obtain may not cover all of the clinical indications for which we are seeking approval, or could contain significant limitations in the form of narrow indications, warnings, precautions or contra-indications with respect to conditions of use. In such event, our ability to generate revenues from such products would be greatly reduced and our business would be harmed.

The FDA has substantial discretion in the approval process and may either refuse to consider our application for substantive review or may form the opinion after review of our data that our application is insufficient to allow approval of our drug candidates. If the FDA does not consider or approve our application, it may require that we conduct additional clinical, preclinical or manufacturing validation studies and submit that data before it will reconsider our application. Depending on the extent of these or any other studies, approval of any applications that we submit may be delayed by several years, or may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be successful or considered sufficient by the FDA to support approval. If any of these outcomes occur, we may be forced to abandon one or more of our applications for approval, which might significantly harm our business, prospects, financial condition and results of operations.

Even if we do receive regulatory approval to market a drug candidate, any such approval may be subject to limitations on the indicated uses for which we may market the product. It is possible that none of our existing drug candidates or any drug candidates we may seek to develop in the future will ever obtain the appropriate

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regulatory approvals necessary for us or our collaborators to commence product sales. Any delay in obtaining, or an inability to obtain, applicable regulatory approvals would prevent us from commercializing our drug candidates, generating revenues and achieving and sustaining profitability.

Even if we obtain and maintain approval for quizartinib from the FDA, we may never obtain approval for quizartinib outside of the United States, which would limit our market opportunities and adversely affect our business.

Sales of quizartinib outside of the United States, if approved, will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products is also subject to approval. We may decide to submit a marketing authorizations application, or MAA to the European Medicines Agency, or EMA, for approval in the European Union. As with the FDA, obtaining approval of an MAA from the EMA is a similarly lengthy and expensive process and the EMA has its own procedures for approval of product candidates. Even if a product is approved, the FDA or the EMA, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of drug candidates with which we must comply prior to marketing in those counties. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of quizartinib will be harmed, which would adversely affect our business, prospects, financial condition and results of operations.

Even if we receive regulatory approval for any of our drug candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our drug candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we or our strategic partners receive for our drug candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-approval commitments, including additional clinical trials and analyses, Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the drug candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our drug candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices, or cGMPs, and current good clinical practices, or

cGCPs, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;

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fines, warning letters or holds on clinical trials;

refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;

product seizure or detention, or refusal to permit the import or export of products; and

injunctions or the imposition of civil or criminal penalties.

The FDA s policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our drug candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we and our collaborators may not be able to obtain regulatory approval for or commercialize our drug candidates and our business could be substantially harmed.

We have agreements with third-party CROs to conduct or monitor and manage data for our ongoing preclinical and clinical programs, including our ongoing clinical trials for quizartinib. We expect to engage additional CROs in connection with our planned Phase 3 clinical trial. We rely heavily on these parties for execution of our preclinical and clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for all of our drug candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA 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, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our or our respective CROs failure to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies or other drug development activities, which could harm our competitive position. If CROs 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 or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize our drug candidates. As a result, our financial results and the commercial prospects for quizartinib and our other drug candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which

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can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, prospects, financial condition and results of operations.

We rely completely on third parties to manufacture our preclinical and clinical drug supplies and we intend to rely on third parties to produce commercial supplies of any approved drug candidate. The development and commercialization of any of our drug candidates, including quizartinib, could be stopped, delayed or made less profitable if those third parties fail to obtain and maintain regulatory approval of their facilities, fail to provide us with sufficient quantities of drug product or fail to do so at acceptable quality levels or prices.

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our drug candidates on a clinical or commercial scale. Instead, we rely on contract manufacturers for the production of quizartinib and our other drug candidates. The facilities used by our contract manufacturers to manufacture our drug candidates must be approved by the applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after an NDA is submitted to the FDA. We do not control the manufacturing process of quizartinib and are completely dependent on our contract manufacturing partners for compliance with the FDA s requirements for manufacture of both the active drug substances and finished quizartinib drug product. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA s strict regulatory requirements, they will not be able to secure or maintain FDA approval for the 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 any other applicable regulatory authorities does not approve these facilities for the manufacture of our drug candidates or if it withdraws any such approval in the future, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture our products, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all, which would significantly impact our ability to develop, obtain regulatory approval for or market our drug candidates.

We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our drug candidates for our clinical trials. There are a small number of suppliers for certain capital equipment and raw materials that we use to manufacture our drugs. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these raw materials. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a drug candidate to complete the clinical trial, any significant delay in the supply of a drug candidate or the raw material components thereof for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our drug candidates. If our manufacturers or we are unable to purchase these raw materials after regulatory approval has been obtained for our drug candidates, the commercial launch of our drug candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our drug candidates.

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if

contaminants are discovered in our products or in the manufacturing facilities in which our products are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability or other issues relating to

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the manufacture of any of our products will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide any drug candidates to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely. In addition, quizartinib has, to date, been dosed as a liquid oral treatment. We have recently developed a solid dosage form (tablet) of quizartinib and successfully completed a Phase 1 clinical trial in healthy volunteers to confirm the relative bioavailability between the liquid and the tablet forms. We anticipate incorporating the tablet in future clinical development, including our planned Phase 3 clinical trial, subject to guidance from the FDA. We may encounter delays in the manufacture of this tablet form, in which case we would need to continue to use the liquid form in future trials. In any event, if approved, our commercial strategy is to have both the tablet form and liquid forms in order to address the needs of multiple patient populations.

Any adverse developments affecting our clinical or commercial manufacturing operations for our products may result in shipment delays, inventory shortages, lot failures, product withdrawals or recalls, or other interruptions in the supply of our products. We may also have to take inventory write-offs and incur other charges and expenses for products that fail to meet specifications, undertake costly remediation efforts or seek more costly manufacturing alternatives. Accordingly, failures or difficulties faced at any level of our supply chain could materially adversely affect our business and delay or impede the development and commercialization of quizartinib or any of our other drug candidates and could have a material adverse effect on our business, prospects, financial condition or results of operations.

We currently do not have the capability to package quizartinib finished drug product for distribution to hospitals and other customers. We have entered into an agreement with a contract manufacturer to supply us with finished product. Prior to commercial launch, we intend to enter into a similar agreement with an alternate fill/finish drug product supplier for quizartinib so that we can ensure proper supply chain management once we are authorized to make commercial sales of quizartinib. Once finalized, we expect that the selected alternate supplier will provide us with finished drug product. If we receive marketing approval from the FDA, we intend to sell drug product finished and packaged by either our current contract manufacturer or this alternate supplier.

We have not entered into long-term agreements with our current contract manufacturers or with any alternate fill/finish suppliers. Although we intend to do so prior to commercial launch of quizartinib in order to ensure that we maintain adequate supplies of finished drug product, we may be unable to enter into such an agreement or do so on commercially reasonable terms, which could have a material adverse impact upon our business and our ability to commercialize quizartinib.

We believe we will have sufficient quantities of manufactured drug substance to support planned development activities. Further, we plan to have our existing contract manufacturers and any alternate suppliers later identified manufacture and package additional bulk drug substance and finished drug product in connection with commercial launch in the event quizartinib is approved for sale by regulatory authorities. If we are unable to do so in a timely manner, the commercial introduction of quizartinib, if approved by the FDA, would be adversely affected.

Obtaining Fast Track designation from the FDA for our drug candidate quizartinib does not guarantee faster approval.

We received Fast Track designation for our drug candidate quizartinib for the treatment of patients 60 years of age or older with FLT3-ITD positive AML in first relapse or refractory to first line chemotherapy and treatment of patients 18 years or older with FLT3-ITD positive AML in second relapse or refractory to second line salvage therapy. Fast Track designation is a process designed to facilitate the development and expedite the

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review of new drugs intended to treat serious or life-threatening diseases or conditions and that have the potential to address an unmet medical need for such disease or condition. Fast Track designation applies to the product and the specific indication for which it is being studied. Once a Fast Track designation is obtained, the FDA may consider for review on a rolling basis sections of the NDA before the complete application is submitted if the applicant provides and the FDA approves a schedule for the submission of the sections of the NDA and the applicant pays applicable user fees upon submission of the first section of the NDA. However, the time period specified in the Prescription Drug User Fee Act, which governs the time period goals the FDA has committed to reviewing an application, does not begin until the complete application is accepted for filing. Although we received Fast Track designation for quizartinib, the FDA may later decide that quizartinib no longer meets the conditions for qualification. In addition, Fast Track designation may not provide us with a material commercial advantage.

We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our drug candidates, we may not be able to generate product revenues.

We currently do not have a commercial organization for the marketing, sales and distribution of pharmaceutical products. In order to commercialize any products, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We contemplate establishing (either internally or through a contract sales force) our own commercial capabilities to market, sell and distribute quizartinib, if approved, in North America and plan to partner with third parties to commercialize quizartinib in other markets.

The establishment and development of our own sales force or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our drug candidates. To the extent we rely on third parties to commercialize our approved products, if any, we may have little or no control over the marketing and sales efforts of such third parties and our revenues from product sales may be lower than if we had commercialized these products ourselves. In the event we are unable to develop our own marketing and sales force or collaborate with a third-party marketing and sales organization, we would not be able to commercialize our drug candidates.

If we fail to develop and commercialize other drug candidates, we may be unable to grow our business.

As a significant part of our growth strategy, we intend to develop and commercialize drug candidates in addition to quizartinib. These other drug candidates will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, extensive clinical trials and approval by the FDA and applicable foreign regulatory authorities. All drug candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the drug candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. In addition, we cannot assure you that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives. A significant portion of the research that we are conducting involves new and unproven technologies. Research programs to identify new drug candidates require substantial technical, financial and human resources whether or not we ultimately identify any candidates. If we are unable to develop our drug candidates, our business and prospects will suffer.

We cannot be certain that our drug candidates will produce commercially viable drugs that safely and effectively treat cancer or other diseases. To date, our technology platform has yielded only a small number of drug candidates other than quizartinib. In addition, we have limited preclinical and clinical data with respect to

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any of these other potential drug candidates. Even if we are successful in completing preclinical and clinical development and receiving regulatory approval for one commercially viable drug for the treatment of one disease, we cannot be certain that we will also be able to develop and receive regulatory approval for other drug candidates for the treatment of other forms of that disease or other diseases. If we fail to develop and commercialize viable drugs, we will not be successful in developing a pipeline of potential drug candidates to follow quizartinib, and our business prospects would be harmed significantly.

Our commercial success depends upon attaining significant market acceptance of our drug candidates, if approved, including quizartinib, among physicians, patients, healthcare payors and, in the cancer market, acceptance by the major operators of cancer clinics.

Even if we obtain regulatory approval for quizartinib or any other drug candidate that we may develop or acquire in the future, the product may not gain market acceptance among physicians, health care payors, patients and the medical community. Market acceptance of quizartinib or any other drug candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety of such drug candidates as demonstrated in clinical trials;
- the clinical indications for which the drug candidate is approved;
- acceptance by physicians, major operators of cancer clinics and patients of the drug as a safe and effective treatment;
- the potential and perceived advantages of drug candidates over alternative treatments;
- the safety of drug candidates seen in a broader patient group, including its use outside the approved indications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- the timing of market introduction of our products as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third party payors and government authorities;

relative convenience and ease of administration; and

the effectiveness of our sales and marketing efforts and those of our collaborators.

If quizartinib or any other drug candidate is approved but fails to achieve market acceptance among physicians, patients, or health care payors, we will not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition and results of operations.

We face significant competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively.

The biotechnology and pharmaceutical industries are intensely competitive. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of

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technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis drug products that are more effective or less costly than quizartinib or any drug candidate that we are currently developing or that we may develop.

Currently there are no approved therapies for relapsed/refractory AML beyond traditional chemotherapy. Quizartinib may face competition in the United States from the off-label use of commercially available kinase inhibitors such as Bayer AG s and Onyx Pharmaceuticals, Inc. s Nexavar® (sorafenib) and Pfizer Inc. s Sutent® (sunitinib), two multi-kinase inhibitors that inhibit FLT3 approved for the treatment of certain solid tumors. However, these multi-kinase inhibitors are not currently approved for the treatment of AML. In addition, several other companies have small molecule and biologic drug candidates in development that target the FLT3 pathway and, if approved, could compete with quizartinib, including Novartis AG s PKC-412 (midostaurin).

Pfizer s Xeljanz® (tofacitinib), a pan-JAK inhibitor, was recently approved in the United States for the treatment of rheumatoid arthritis, and several companies have inhibitors of the JAK family of kinases in clinical development for inflammatory disease. A number of companies have oral small molecule and biologic colony-stimulating factor-1 receptor, or CSF1R, inhibitors in clinical development. Daiichi-Sankyo Company Limited s, and F. Hoffman-LaRoche Ltd s Zelboraf® (vemurafenib), a BRAF kinase inhibitor, was approved by the FDA in 2011 for the treatment of metastatic melanoma patients harboring the V600E BRAF mutation, and several other companies have BRAF inhibitors in clinical development.

Our ability to compete successfully will depend largely on our ability to leverage our experience in drug discovery and development to:

- discover and develop highly selective and potent small molecule drugs that inhibit validated kinase targets and that are superior to other products in the market;
- attract qualified scientific, product development and commercial personnel;
- obtain patent and/or other proprietary protection for our medicines and technologies;
- obtain required regulatory approvals; and
- successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability and price of our competitors products could limit the demand, and the price we are able to charge, for quizartinib or any of our other drug candidates, if approved. We will not achieve our business plan if the acceptance of quizartinib is inhibited by price competition or the reluctance of physicians to switch from existing drug products to quizartinib, or if physicians switch to other new drug products or choose to reserve quizartinib for use in limited circumstances. The inability to compete with existing or subsequently introduced drug products would have a material adverse impact on our business, prospects, financial condition and results of operations.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our drug candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA approval or discovering, developing and commercializing medicines before we do, which would have a material adverse impact on our business.

Reimbursement may be limited or unavailable in certain market segments for our drug candidates, which could make it difficult for us to sell our products profitably.

We intend to seek approval to market quizartinib in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for quizartinib or any of our other drug

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candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of prescription pharmaceuticals and biologics 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 candidate. In addition, market acceptance and sales of our drug candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for any of our drug candidates and may be affected by existing and future health care reform measures.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will pay for and establish reimbursement levels. Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor s determination that use of a product is:

a covered benefit under its health plan;

safe, effective and medically necessary;

appropriate for the specific patient;

cost-effective; and

neither experimental nor investigational.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If reimbursement of our future products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability.

In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, the Medicare Modernization Act of 2003 revised the payment methodology for many products under Medicare in the United States. This has resulted in lower rates of reimbursement. In 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, collectively, the Healthcare Reform Act, was enacted. The Healthcare Reform Act contains a number of provisions, including those governing enrollment in federal healthcare programs, the increased use of comparative effectiveness research on healthcare products, reimbursement and fraud and abuse changes, which will impact existing government healthcare programs and will result in the development of new programs. An expansion in the government s role in the U.S. healthcare industry may further lower rates of reimbursement for pharmaceutical products.

We cannot predict whether legal challenges will result in changes to the Healthcare Reform Act or if other legislative changes will be adopted, or how such changes would affect our business. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers.

There have been, and likely will continue to be, legislative and regulatory proposals at the federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any drug candidates for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;

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our ability to generate revenues and achieve or maintain profitability;

the level of taxes that we are required to pay; and

the availability of capital.

In addition, governments may impose price controls, which may adversely affect our future profitability.

We may form strategic alliances in the future, and we may not realize the benefits of such alliances.

We may form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties that we believe will complement or augment our existing business, including with respect to quizartinib and our JAK2 and CSF1R programs. These relationships or those like them may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for quizartinib or our JAK2 and CSF1R programs or any future drug candidates and programs because our research and development pipeline may be insufficient, our drug candidates and programs may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our drug candidates and programs as having the requisite potential to demonstrate safety and efficacy. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenues or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our drug candidates could also delay the development and commercialization of our drug candidates and reduce their competitiveness even if they reach the market.

If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, including our President and Chief Executive Officer, Michael A. Martino, our Chief Medical Officer, Athena Countouriotis, M.D., and our Chief Financial Officer, Alan Fuhrman. In order to induce valuable employees to remain at Ambit, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Our scientific team has expertise in many different aspects of drug discovery and development. We conduct our operations at our facility in San Diego, California. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is very intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with all of our employees, these employment agreements provide for at-will employment, which means that any of our employees

could leave our employment at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations. We do not maintain key man insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

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Many of the other biotechnology and pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high quality candidates than what we can offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize drug candidates will be limited.

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

As of December 31, 2013, we employed 53 employees, 49 of whom were full-time. As our development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional managerial, operational, clinical, regulatory, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical and FDA review process for quizartinib and our other drug candidates, while complying with our contractual obligations to licensors, licensees, contractors, collaborators and third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize quizartinib and other drug candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. To date, we have used the services of outside vendors to perform tasks including clinical trial management, statistics and analysis, regulatory affairs, formulation development and other drug development functions. Our growth strategy may also entail expanding our group of contractors or consultants to implement these tasks going forward. Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for quizartinib and our other drug candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our drug candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors and consultants and collaborators are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have

not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties to manufacture our drug candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our drug candidates could be delayed.

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Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce our drug candidates. Our ability to obtain clinical supplies of quizartinib or our other drug candidates could be disrupted, if the operations of these suppliers is affected by a man-made or natural disaster or other business interruption. Our corporate headquarters is located in California near major earthquake faults and fire zones. The ultimate impact on us, our significant suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

A variety of risks associated with marketing our drug candidates internationally could materially adversely affect our business.

If approved for commercialization in the United States, we also expect to seek approval to market quizartinib outside of the United States. Consequently, we expect that we will be subject to additional risks related to operating in foreign countries including:

- differing regulatory requirements for drug approvals 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;
- 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 revenues, 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 materially adversely affect our ability to attain or maintain profitable operations.

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Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards we have established, to comply with federal and state health-care fraud and abuse laws and regulations, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct and Ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to 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 fines or other sanctions.

We may also be subject to healthcare laws, regulation and enforcement; our failure to comply with those laws could have a material adverse effect on our results of operations and financial conditions.

Although we currently do not have any products on the market, if any of our drug candidates are approved, once we begin commercializing our products, we may be subject to additional healthcare regulation and enforcement by the federal government and the states and foreign governments in which we conduct our business. The laws that may affect our ability to operate include, without limitation, state and federal anti-kickback, false claims, privacy and security and physician sunshine laws and regulations. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our drug candidates.

We face an inherent risk of product liability as a result of the clinical testing of our drug candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if any drug candidate we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our drug candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

decreased demand for our drug candidates or products that we may develop;

injury to our reputation;

withdrawal of clinical trial participants;

initiation of investigations by regulators;

costs to defend the related litigation;

a diversion of management s time and our resources;

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substantial monetary awards to trial participants or patients;

product recalls, withdrawals or labeling, marketing or promotional restrictions;

loss of revenue;

exhaustion of any available insurance and our capital resources;

the inability to commercialize our drug candidates; and

a decline in our share price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry $10.0 million of product liability insurance covering our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. If we determine that it is prudent to increase our product liability coverage due to the commercial launch of any approved product, we may be unable to obtain such increased coverage on acceptable terms, or at all. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

If we use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials. In addition, our operations produce hazardous waste products and those of our manufacturers and some CROs may produce medical and radioactive waste products. We and our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our and our manufacturers procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we may incur significant additional costs to comply with applicable laws in the future. Also, even if we are in compliance with applicable laws, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

Risks Related to Our Financial Position and Capital Requirements

We have a limited operating history, have incurred significant operating losses since our inception and anticipate that we will continue to incur losses for the foreseeable future.

Our operations began in 2000 and we have only a limited operating history upon which you can evaluate our business and prospects. Our operations to date have been limited to conducting product development activities for quizartinib and other drug candidates and performing research and development with respect to our clinical and preclinical programs. In addition, as an early stage company, we have limited experience and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical area. Nor have we demonstrated

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an ability to obtain regulatory approval for or to commercialize a drug candidate. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing pharmaceutical products.

We have incurred significant operating losses since our inception, including consolidated net losses of $11.3 million, $27.0 million and $37.4 million for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, we had an accumulated deficit of $248.2 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders equity and working capital. Our losses have resulted principally from costs incurred in our discovery and development activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our discovery, research, development and commercialization activities, including the clinical development and planned commercialization of our lead drug candidate, quizartinib, and incur the additional costs of operating as a public company. In addition, if we obtain regulatory approval of quizartinib, we may incur significant sales and marketing expenses. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

We have limited sources of revenues and have not generated any revenues to date from product sales. We may never achieve or sustain profitability, which could depress the market price of our common stock, and could cause you to lose all or a part of your investment.

Our ability to become profitable depends on our ability to develop and commercialize quizartinib and our other drug candidates. To date, we have no products approved for commercial sale and have not generated any revenues from sales of any drug candidate, and we do not know when, or if, we will generate revenues in the future. Substantially all of our revenues to date have come from research service fees, license or collaboration agreements and our screening business, which we sold in October 2010. We do not anticipate generating revenues, if any, from sales of quizartinib for at least the next several years and we will never generate revenues from quizartinib if we and Genoptix do not obtain regulatory approval for quizartinib and its companion diagnostic, respectively. Our ability to generate future revenues depends heavily on our and our collaborators success in:

- developing and securing U.S. and/or foreign regulatory approvals for quizartinib and its companion diagnostic;
- manufacturing commercial quantities of quizartinib at acceptable cost;
- achieving broad market acceptance of quizartinib in the medical community and with third-party payors and patients;
- commercializing quizartinib and any other drug candidates for which we receive approval;
- pursuing clinical development of quizartinib in additional indications, as well as clinical development of other drug candidates; and

generating a pipeline of innovative drug candidates using our drug discovery platform or through licensing strategies.

Even if we do generate product sales, we may never achieve or sustain profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations.

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

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. 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 may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or drug candidates, or grant licenses on terms unfavorable to us.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an ownership change (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 believe that, as a result of our initial public offering, or IPO, the concurrent private placement and other transactions that have occurred over the past three years, we have experienced an ownership change. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As of December 31, 2013, we had federal and state net operating loss carryforwards of approximately $192.8 million and $181.7 million, respectively, and federal research and development credits of $6.4 million which could be limited if we experience an ownership change.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As widely reported, global credit and financial markets have experienced extreme disruptions in the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

At December 31, 2013, we had $71.2 million of cash and cash equivalents. While we are not aware of any downgrades, material losses, or other significant deterioration in the fair value of our cash equivalents since December 31, 2013, no assurance can be given that further deterioration of the global credit and financial markets would not negatively impact our current portfolio of cash equivalents or marketable securities or our ability to meet our financing objectives. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

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Risks Related to Our Intellectual Property

If our efforts to protect the proprietary nature of the intellectual property related to our technologies are not adequate, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies. Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market.

Composition-of-matter patents on the active pharmaceutical ingredient are generally considered to be the strongest form of intellectual property protection for pharmaceutical products, as such patents provide protection without regard to any method of use. We cannot be certain that the claims in our patent applications covering composition-of-matter of our drug candidates will be considered patentable by the United States Patent and Trademark Office, or the U.S. PTO, courts in the United States, or by the patent offices and courts in foreign countries. Method-of-use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products off-label. Although off-label prescriptions may infringe or contribute to the infringement of method-of-use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or license may fail to result in issued patents in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our patents and patent applications, including those that we license to Teva, may not adequately protect our intellectual property or prevent others from designing around our claims. If the breadth or strength of protection provided by the patent applications we hold with respect to quizartinib or the patents we hold or pursue with respect to other drug candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our drug candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our drug candidates under patent protection would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to file any patent application related to quizartinib or our other candidates. Furthermore, for applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the U.S. PTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. For applications containing a claim not entitled to priority before March 16, 2013, there is greater level of uncertainty in the patent law with the passage of the America Invents Act (2012) which brings into effect significant changes to the U.S. patent laws that are yet untried and untested, and which introduces new procedures for challenging pending patent applications and issued patents. A primary change under this reform is creating a first to file system in the U.S. This will require us to be cognizant after March 16, 2013 of the time from invention to filing of a patent application.

In addition to the protection afforded by patents, we seek to rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. Although we require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot be certain that

our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop

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substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

Third-party claims of intellectual property infringement may prevent or delay our drug discovery and development efforts.

Our commercial success depends in part on our and our collaborators avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the U.S. PTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including inter partes review and post grant review have been implemented or will be implemented as of March 16, 2013. As stated above, this reform is untried and untested and will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing drug candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our drug candidates may give rise to claims of infringement of the patent rights of others.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of quizartinib and/or our other drug candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our drug candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our drug candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such drug candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the applicable drug candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. We are aware of a third party patent that relates to an inactive ingredient that we currently use in quizartinib, as well as a third party patent related to diagnostic testing for certain FLT3 mutations in patient samples. Should a license to either third party patent become necessary, we cannot predict whether we or our partners would be able to obtain a license to either of the above, or if a license were available, whether it would be available on commercially reasonable terms. If such patents have a valid claim relating to our use of the inactive ingredient or diagnostic testing required to detect FLT3 mutations and, in either case, a license under the applicable patent is unavailable on commercially reasonable terms, or at all, our ability to commercialize quizartinib may be impaired or delayed, which could in turn significantly harm our business.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our drug candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of

employee resources from our business. 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,

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obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our drug candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our drug candidates, which could harm our business significantly.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. 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, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

Interference proceedings provoked by third parties or brought by the U.S. PTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our collaborators or licensors. 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. Litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

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. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. 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.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the U.S. PTO and foreign patent agencies in several stages over the lifetime of the patent. The U.S. PTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant

jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to

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official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties, including entities that disclosed such information to us in connection with previously provided screening services.

We have received confidential and proprietary information from collaborators, prospective licensees and other third parties. In addition, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies. We may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of these third parties or our employees former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial cost and be a distraction to our management and employees.

Risks Related to Ownership of our Common Stock

The price of our stock may be volatile, and you could lose all or part of your investment.

Prior to our IPO, there was no public market for our common stock. Since our IPO in May 2013, the trading price of our common stock has ranged from a low of approximately $6 to a high of approximately $21. The trading price of our common stock is likely to continue to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this Risk Factors section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our planned Phase 3 clinical trial of quizartinib or any ongoing or future clinical trials we may conduct, or changes in the development status of quizartinib or any other drug candidate;
- any delay in filing our NDA for quizartinib and any adverse development or perceived adverse development with respect to the FDA s review of the NDA, including without limitation the FDA s issuance of a refusal to file letter or a request for additional information;
- adverse results or delays in clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for quizartinib or the companion diagnostic;

changes in laws or regulations applicable to our products, including but not limited to clinical trial requirements for approvals;

adverse developments concerning our collaborations and our manufacturers;

inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;

the inability to establish additional collaborations;

our failure to commercialize quizartinib and the companion diagnostic, develop additional drug candidates and commercialize additional drug products;

additions or departures of key scientific or management personnel;

unanticipated serious safety concerns related to the use of quizartinib or any of our other drug candidates;

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introduction of new products or services offered by us or our competitors;

announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;

our ability to effectively manage our growth;

the size and growth, if any, of the AML market and other targeted markets;

our ability to successfully enter new markets or develop additional drug candidates;

actual or anticipated variations in quarterly operating results;

our cash position;

failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;

publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;

changes in the market valuations of similar companies;

overall performance of the equity markets;

issuances of debt or equity securities;

sales of our common stock by us or our stockholders in the future;

trading volume of our common stock;

changes in accounting practices;

ineffectiveness of our internal controls;

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, the stock market in general, and the Nasdaq Global Market and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company s securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management s attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, any future debt financing arrangement may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited to the appreciation of their stock. Investors seeking cash dividends should not invest in our common stock.

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Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of December 31, 2013, our executive officers, directors, 5% stockholders and their affiliates owned approximately 65% of our voting stock. Therefore, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders. Furthermore, as a thinly-traded stock, if any of our major stockholders decide to liquidate their holdings, for whatever reason, the impact on our stock price could be detrimental over a prolonged period of time.

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock 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. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also 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, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

The requirements of being a public company may strain our resources and divert our management s attention.

As a public company, we have incurred, and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted

by the SEC, and the Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010,

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the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and proxy access. Recent legislation permits smaller emerging growth companies to implement many of these requirements over a longer period and up to five years from the IPO. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our consolidated net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, our stockholders may be materially diluted by subsequent sales, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

Pursuant to our 2013 equity incentive plan, or 2013 post-IPO plan, our management is authorized to grant stock options to our employees, directors and consultants. The number of shares available for future grant under our 2013 post-IPO plan will automatically increase each year by an amount equal to 4% of all shares of our capital stock outstanding as of January 1st of each year, subject to the ability of our board of directors to take action to reduce the size of such increase in any given year. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

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

Provisions in our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third-party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management. These provisions include:

authorizing the issuance of blank check preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;

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limiting the removal of directors by the stockholders;

creating a staggered board of directors;

prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;

eliminating the ability of stockholders to call a special meeting of stockholders;

permitting our board of directors to accelerate the vesting of outstanding option grants upon certain transactions that result in a change of control; and

establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management. We are also subject to certain anti-takeover provisions under Delaware law which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under Delaware law, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our certificate of incorporation or bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Item 1B. Unresolved Staff Comments.

Not applicable.

Item 2. Properties.

We currently lease approximately 20,000 square feet of laboratory and office space at 11080 Roselle Street, San Diego, California, 92121. Our lease for this facility expires in September 2018. Our current monthly lease obligation is approximately $52,000. We are responsible for expenses associated with the use and maintenance of the building, such as utilities and maintenance. In addition, the lease is subject to additional charges for property management, common area maintenance and other costs. These costs will vary each month, and we expect that these costs will be approximately $27,000 per month.

We believe that our facility is sufficient to meet our needs and that suitable additional space will be available as and when needed.

Item 3. Legal Proceedings.
None.

Item 4. Mine Safety Disclosures.
Not applicable.

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PART II

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

Market Information

Our common stock began trading on The NASDAQ Global Market on May 16, 2013 under the symbol AMBI. Prior to such time, there was no public market for our common stock. The following table sets forth the high and low sales prices per share of our common stock as reported on The NASDAQ Global Market for the period indicated.

	High	Low
Year Ended December 31, 2013		
Second Quarter (commencing May 16, 2013)	$ 8.10	$ 6.22
Third Quarter	$ 17.11	$ 7.01
Fourth Quarter	$ 21.44	$ 7.77

Holders of Record

As of February 28, 2014, there were approximately 86 holders of record of our common stock.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future. Any future determination related to dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about our equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

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Performance Graph

The following graph shows a comparison from May 16, 2013 (the date our common stock commenced trading on The NASDAQ Global Market) through December 31, 2013 of the cumulative total return for our common stock, the NASDAQ Composite Index (CCMP) and the NASDAQ Biotechnology Index (NBI). The graph assumes an initial investment of $100 on May 16, 2013. The comparisons in the graph are not intended to forecast or be indicative of possible future performance of our common stock.

Recent Sales of Unregistered Securities

During the fiscal year ended December 31, 2013, we issued and sold the following unregistered securities (excluding those previously disclosed in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K):

In January 2013, in connection with our Series E preferred stock financing, Ambit Biosciences (Canada) Corporation, or Ambit Canada, issued and sold 3,916,693 shares of its Class E preferred shares to GrowthWorks Canadian Fund Ltd. at a purchase price of $0.70 per share, for aggregate gross proceeds of $2.7 million. These shares converted into 163,195 shares of our common stock on the completion of our IPO.

The sales and issuances of securities in the transactions described above were deemed to be exempt from registration under the Securities Act in reliance upon Rule 701 promulgated under Section 3(b) of the Securities Act as transactions pursuant to compensatory benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of securities in each transaction represented their intentions to acquire 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. All recipients had adequate access,

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through employment or other relationships, to information about us. All certificates representing the securities issued in these transactions included appropriate legends setting forth that the securities had not been offered or sold pursuant to a registration statement and describing the applicable restrictions on transfer of the securities.

Use of Proceeds

On May 15, 2013, we commenced our IPO pursuant to a registration statement on Form S-1 (File No. 333-186760) that was declared effective by the SEC on May 15, 2013 and that registered an aggregate of 9,343,750 shares of our common stock for sale to the public at a price of $8.00 per share at an aggregate offering price of $74,750,000. On May 21, 2013, we sold 8,125,000 shares of our common stock to the public at a price of $8.00 per share for an aggregate gross offering price of $65,000,000.

Upon receipt, the net proceeds from our IPO were invested in cash equivalents. As of December 31, 2013, we estimate that we had used approximately $167,000 for the purchase of equipment and approximately $20.7 million for working capital expenditures. We intend to use the remainder of the net proceeds from the IPO to fund the continued clinical development of quizartinib, to fund the continued development of our other programs and for working capital and other general corporate purposes. The amounts and timing of our actual expenditures depend on numerous factors, including the ongoing status of and results from clinical trials and other studies, as well as any strategic partnerships that we may enter into with third parties for our drug candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from our IPO and the concurrent private placement and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our stock.

Issuer Repurchases of Equity Securities

None.

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Item 6. Selected Consolidated Financial Data.

The following selected financial data should be read together with our consolidated financial statements and accompanying notes and information under the caption Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this Annual Report on Form 10-K. The selected consolidated financial data in this section are not intended to replace our consolidated financial statements and the related notes. Our historical results are not necessarily indicative of results that may be expected in the future.

The selected consolidated statement of operations data for the years ended December 31, 2013, 2012 and 2011 and the selected consolidated balance sheet data as of December 31, 2013 and 2012 are derived from our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

	Year Ended December 31,		
	2013	2012	2011
	(in thousands, except share and per share data)		
Consolidated Statement of Operations Data:			
Revenues:			
Collaboration agreements	$ 27,093	$ 17,633	$ 23,843
Operating expenses:			
Research and development	26,284	36,731	50,705
General and administrative	10,342	6,550	8,905
Gain on sale of kinase profiling services business	(2,500)	(2,497)	(2,108)
Total operating expenses	34,126	40,784	57,502
Loss from operations	(7,033)	(23,151)	(33,659)
Other income (expense):			
Interest expense	(323)	(1,737)	(4,502)
Other income (expense)	143	29	1,538
Change in fair value of warrant and derivative liabilities	(4,072)	(2,291)	(795)
Total other income (expense)	(4,252)	(3,999)	(3,759)
Loss before income taxes	(11,285)	(27,150)	(37,418)
Benefit before income taxes	(29)	(121)	
Consolidated net loss	(11,256)	(27,029)	(37,418)
Net loss (income) attributable to redeemable non-controlling interest	61	382	(213)
Net loss attributable to Ambit Biosciences Corporation	(11,195)	(26,647)	(37,631)
Accretion to redemption value of redeemable convertible preferred stock	(3,634)	(3,161)	(2,000)
Change in fair value of redeemable non-controlling interest	1,747	(854)	4,477
Net loss attributable to common stockholders	$ (13,082)	$ (30,662)	$ (35,154)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (1.19)	$ (16,591.99)	$ (25,886.60)

Weighted average shares outstanding, basic and diluted[(1)]	11,024,175	1,848	1,358

(1) Please see Note 1 to our consolidated financial statements for an explanation of the method used to calculate the historical net loss per share attributable to common stockholders, basic and diluted, and the number of shares used in computation of the per share amounts.

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	As of December 31,		
	2013	2012	2011
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 71,189	$ 17,481	$ 16,417
Working capital (deficit)	56,742	(11,113)	(6,023)
Total assets	73,948	19,989	22,820
Notes payable, net of debt discount		4,320	8,911
Warrant liabilities	9,650	10,540	4,916
Redeemable non-controlling interest		3,323	1,322
Redeemable convertible preferred stock		157,076	132,340
Convertible preferred stock		13,702	13,752
Accumulated deficit	(248,166)	(236,971)	(210,324)
Total stockholders equity (deficit)	57,590	(198,246)	(177,364)

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

You should read the following discussion and analysis together with Item 6. Selected Financial Data and our financial statements and related notes included elsewhere in this Annual Report. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including those set forth under the caption Item 1A. Risk Factors.

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of drugs to treat unmet medical needs in oncology, autoimmune and inflammatory diseases by inhibiting kinases that are important drivers for those diseases. Our pipeline currently includes three programs, each discovered internally and each aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, is a once-daily, orally administered FMS-like tyrosine kinase 3, or FLT3, kinase inhibitor. We are planning to initiate a Phase 3 clinical trial of quizartinib in the second quarter of 2014 in patients with acute myeloid leukemia, or AML, who express a genetic mutation in FLT3 (referred to by us as FLT3-ITD positive) who are refractory to or relapsed after first-line treatment with or without hematopoietic stem cell transplantation, or HSCT, consolidation. Under our proposed study design, we plan to enroll approximately 326 patients who will be randomized 2:1 to quizartinib monotherapy or salvage chemotherapy and a single interim analysis is planned. Our second drug candidate in clinical development, AC410, is a potent, selective, orally-administered, small molecule inhibitor of Janus kinase 2, or JAK2, that has potential utility for the treatment of autoimmune and inflammatory diseases. Our third program consists of a potent and exquisitely selective small molecule compound, AC708, which inhibits the colony-stimulating factor-1 receptor, or CSF1R, a receptor tyrosine kinase. This compound is in preclinical studies and has potential utility in oncology, autoimmune and inflammatory diseases. All of our drug candidates and clinical candidates have been internally discovered by us.

We were incorporated in Delaware and commenced operations in 2000. Since 2005, most of our activities have related to the research and development of our drug candidates. Prior to 2005, we were focused on the development of a kinase screening platform and services related to that platform. In order to focus on drug discovery and development, in October 2010 we sold all of the assets relating to our kinase profiling services business to DiscoveRx Corporation, pursuant to an asset purchase agreement. As part of the agreement, we made a $5.5 million aggregate commitment to purchase screening services in fiscal years ending December 31, 2011 and December 31, 2012, with payments of approximately $625,000 during each full calendar quarter during such periods. As a result of the commitment, we deferred $5.5 million of the gain on the sale transaction. In this transaction we acquired from DiscoveRx a non-exclusive, worldwide, sublicensable and royalty-free license to the intellectual property related to our former kinase profiling services business, as such intellectual property rights existed as of the date of the sale to DiscoveRx.

We have no products approved for sale, we have not generated any revenues from product sales and we have incurred significant operating losses since our inception. We have generated revenues from upfront payments and reimbursements associated with our collaboration agreements and from our former kinase profiling services business. We have never been profitable and have incurred consolidated net losses of approximately $11.3 million, $27.0 million and $37.4 million in the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, we had an accumulated deficit of $248.2 million.

We expect to continue to incur significant operating losses and negative cash flows from operating activities for the foreseeable future as we continue the clinical development of quizartinib, seek regulatory approval for and, if approved, pursue eventual commercialization of quizartinib, and advance our other drug candidates through preclinical studies and clinical trials. As of December 31, 2013, we had cash and cash equivalents of $71.2 million. Although it is difficult to predict future liquidity requirements, we believe that our existing cash and cash equivalents,

together with interest thereon, will be sufficient to fund our operations through at least the next 12 months. However, successful transition to profitability is dependent upon achieving a level of revenues

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adequate to support our cost structure. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities and, unless and until we do, we will need to raise substantial additional capital through debt or equity financings or through collaborations or partnerships with other companies. We may not be able to raise additional capital on terms acceptable to us, or at all, and any failure to raise capital as and when needed could have a material adverse effect on our results of operations, financial condition and our ability to execute on our business plan.

We conduct the majority of our activities through Ambit Biosciences Corporation, a Delaware corporation, from our primary facility in San Diego, California. Additionally, we have two wholly-owned subsidiaries, Ambit Canada and Ambit Europe Limited. Ambit Canada has in the past conducted limited research and development activities in Toronto. Ambit Europe Limited is located in the United Kingdom and has limited operations related to regulatory filings in the European Union. The following information is presented on a consolidated basis to include the accounts of these subsidiaries. All intercompany transactions and balances are eliminated in consolidation.

In May 2013, we completed our initial public offering, or IPO, of common stock pursuant to a registration statement on Form S-1 that was declared effective on May 15, 2013. In the IPO, we sold 8,125,000 shares of our common stock at a price of $8.00 per share. As a result of the IPO, we raised a total of $58.1 million in net proceeds after deducting underwriting discounts and commissions of approximately $4.6 million and offering expenses of approximately $2.3 million. Costs directly associated with the IPO were capitalized and recorded as deferred IPO costs prior to the closing of the IPO. These costs have been recorded as a reduction of the proceeds received in arriving at the amount to be recorded as additional paid-in-capital. In addition, in connection with the completion of the IPO, all outstanding convertible preferred stock converted into 6,449,073 shares of common stock.

Concurrent with the closing of the IPO, we sold 3,134,495 shares of common stock to certain of our existing shareholders in a concurrent private placement at the IPO price of $8.00 per share and received net proceeds of $25.1 million.

Collaboration Agreements

Astellas

In December 2009, we entered into a worldwide agreement with Astellas Pharma Inc., or Astellas, to jointly research, develop and commercialize certain FLT3 kinase inhibitors. As partial consideration for the exclusive license rights granted to Astellas, we received an upfront payment of $40.0 million. Under the agreement, we and Astellas shared equally in agreed-upon development and research costs in the United States and European Union for quizartinib and certain designated follow-on compounds to quizartinib through the effective date of the termination. In March 2013, we received a notice of termination of the agreement from Astellas, which termination was effective in September 2013. We now own all rights to quizartinib and any follow-on compounds and are responsible for all development and commercialization activities and related costs in the United States, Europe and the rest of the world. We are planning to seek strategic partnerships to pursue development and commercialization activities of quizartinib outside of North America.

Teva

In November 2006, we entered into an exclusive collaboration agreement with Cephalon, Inc., aimed at identifying and developing clinical candidates that demonstrate activity towards the two designated target kinases of the collaboration: the BRAF kinase and a second kinase determined by a joint research committee. Under the agreement, both parties contributed certain intellectual property to the collaboration and agreed to a period of exclusivity during which neither party would engage in any research related to a collaboration target compound with any third-party. In

October 2011, Teva Pharmaceutical Industries Ltd., or Teva, acquired Cephalon, Inc.

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Cephalon, Inc. paid us an upfront fee of $15.5 million as partial consideration for access to our profiling technology and the licenses we contributed to the collaboration. We have earned three milestone payments totaling $4.0 million under the agreement to date and we may be entitled to receive up to $44.5 million in additional payments upon the achievement of development, regulatory and sales milestones for CEP-32496, and up to $46.5 million in payments upon the achievement of development, regulatory and sales milestones for the second compound under the agreement. In addition, we may receive tiered royalty payments ranging from the mid-single digits to the low double digits calculated as a percentage of net sales of the collaboration compounds, including CEP-32496, subject to certain offsets. Royalties are payable to us on a product-by-product, country-by-country basis beginning on the date of the first commercial sale in a country and ending on the later of 10 years after the date of such sale in that country or the expiration date of the last to expire patent covering the licensed product in that country. The collaboration portion of the agreement ended in November 2009, at which point we had completed all our research obligations under the agreement. The agreement remains in effect on a product-by-product, country-by-country basis until all royalty obligations expire.

Genoptix

In September 2010, we entered into a collaboration agreement with Genoptix, Inc. to develop a laboratory diagnostic test to identify patients that harbor ITD mutations in their FLT3 receptor tyrosine kinase. Genoptix, Inc. was subsequently acquired by Novartis AG and Genoptix Medical Laboratory, a Novartis company, or Genoptix, assumed all rights and responsibilities of the agreement. Under this agreement, Genoptix will contribute its expertise in developing laboratory tests and we will supply certain patient samples to the collaboration. Genoptix has the right to commercialize the approved test. We will initially pay for the development activities under the collaboration and pursuant to an agreed-upon budget, and are entitled to single-digit royalty payments from Genoptix until we have recouped the development costs plus an additional predetermined percentage of such costs. We intend for this test to be approved by the FDA as a companion diagnostic test in concert with quizartinib. We believe the FDA approval of this test will satisfy the FDA s requirement that a companion diagnostic test be approved with quizartinib.

Financial Overview

Revenues

We have generated revenues from upfront, milestone and collaborative research activity payments received under our collaboration agreements. Reimbursements paid to us from Astellas for 50% of the eligible research and development costs incurred by us under our collaboration agreement are recorded as revenue. Any amounts due to Astellas for our share of costs incurred by Astellas are recorded as research and development costs.

We currently have no products approved for sale, and we have not generated any revenues from product sales or product royalties and do not expect to receive any revenues from any drug candidates unless and until they obtain regulatory approval. To date, we have not submitted any drug candidate for regulatory approval. In the future, we may generate revenues from a combination of additional milestone payments, reimbursements, and royalties in connection with our existing and any future collaborations, as well as product sales for any approved products. However, other than potential milestone payments from Teva, we do not expect to receive revenues unless and until we receive approval for quizartinib or potentially enter into additional collaboration agreements for quizartinib or our other drug candidates. If we fail to achieve clinical success in the development of quizartinib in a timely manner and/or obtain regulatory approval for this drug candidate, our ability to generate future revenues would be materially adversely affected.

Research and Development Expenses

The majority of our operating expenses to date have been incurred in research and development activities. Research and development expenses relate primarily to the discovery and development of our drug candidates. Our business model is dependent upon our continuing to conduct a significant amount of research and development. To date, quizartinib represents the largest portion of our research and development expense. From

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the date of our agreement with Astellas and through the effective date of the termination, we share equally in any agreed-upon research and development costs for quizartinib and any follow-on compounds in the United States and European Union and Astellas is solely responsible for development costs outside of the United States and European Union. Following the effective date of the termination, we will be responsible for all world-wide development costs for quizartinib and any follow-on compounds. Our research and development expenses consist primarily of:

- expenses incurred under agreements with contract research organizations, or CROs, investigative sites and consultants that conduct our clinical trials and a substantial portion of our preclinical studies;
- employee-related expenses, which include salaries and benefits;
- the cost of developing our chemistry, manufacturing and controls capabilities, or CMC, and acquiring clinical trial materials;
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities and equipment, and depreciation of fixed assets;
- stock-based compensation expense to employees and consultants; and
- costs associated with other research activities and regulatory approvals.

Research and development costs are expensed as incurred.

The following table indicates our research and development expense by project/category for the periods indicated (in thousands):

	Year Ended December 31,			Total January 1, 2007 through December 31,
	2013	2012	2011	2013
Quizartinib	$ 18,188	$ 26,880	$ 35,491	$ 123,614
AC410 /AC430	140	792	3,723	15,763
CSF1R	2,593	1,440	2,739	13,922
Discovery projects	2,277	4,625	4,866	60,820
R&D administration	3,086	2,994	3,886	16,498
Total	$ 26,284	$ 36,731	$ 50,705	$ 230,617

Prior to 2007, we did not track research and development costs by project/category.

At this time, due to the inherently unpredictable nature of preclinical and clinical development and given the early stage of our clinical and preclinical programs, we are unable to estimate with any certainty the costs we will incur in the continued development of quizartinib and our other clinical and preclinical programs. Clinical development timelines, the probability of success and development costs can differ materially from expectations. While we are currently focused on advancing quizartinib, our future research and development expenses will depend on the preclinical and clinical success of each drug candidate that we develop, as well as ongoing assessments of the commercial potential of such drug candidates. In addition, we cannot forecast with any degree of certainty which drug candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements.

Research and development expenditures will continue to be significant and will increase as we continue development of quizartinib and advance the development of our proprietary pipeline of novel drug candidates over at least the next several years. We expect to incur significant research and development costs as we complete the ongoing clinical trials of quizartinib, conduct our planned Phase 3 clinical trial in relapsed/refractory AML patients, which we plan to initiate in early 2014, subject to receiving input from regulatory authorities, and prepare regulatory submissions.

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The costs of clinical trials may vary significantly over the life of a project owing to factors that include but are not limited to the following:

- per patient trial costs;
- the number of patients that participate in the trials;
- the number of sites included in the trials;
- the countries in which the trial is conducted;
- the length of time required to enroll eligible patients;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient follow-up; and
- the efficacy and safety profile of the drug candidate.

We do not expect quizartinib to be commercially available, if at all, for at least the next several years. We base our expenses related to clinical trials on estimates which are based on our experience and estimates from CROs and other third parties.

Sale of Kinase Profiling Services Business

On October 21, 2010, we sold all of the assets relating to our kinase profiling service business to DiscoveRx Corporation, or DiscoveRx, pursuant to an asset purchase agreement. In consideration for the sale of such assets, DiscoveRx paid us $7.3 million at the closing of the transaction, $0.4 million in January 2011 and may be required to pay us up to an additional $4.5 million of incremental consideration upon the achievement by DiscoveRx of certain sales and operational milestones. Under the terms of the asset purchase agreement, we were obligated to purchase from DiscoveRx a minimum of $625,000 of screening services during each full calendar quarter through December 31, 2012. To the extent minimum quarterly commitments exceeded the actual amount of services received, we paid the difference, which was accounted for as a reduction in both the sales price and the overall gain recorded on the sale of the business. In August 2013, we were notified that DiscoverRx has achieved certain, but not all, specified

sales and operational milestones, resulting in our earning $2.5 million of the $4.5 million of incremental consideration. We are no longer eligible to earn the remaining $2.0 million of incremental consideration.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for personnel in executive, finance, business development, marketing, and legal functions. Other general and administrative expenses include facility costs, patent filing costs, and professional fees for legal, consulting, auditing and tax services.

We anticipate that our general and administrative expenses will continue to be significant and will increase as a result of being a public company and associated increased payroll, expanded infrastructure and higher consulting, legal, accounting and investor relations costs, and director and officer insurance premiums.

In addition, we expect to incur increased expenses associated with building a sales and marketing team. We expect to start incurring such expenses prior to receiving regulatory approval of quizartinib. We do not expect to receive any such regulatory approval for at least the next several years.

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Interest Expense

Interest expense consists primarily of coupon interest, amortization of debt discount and amortization of deferred financing costs associated with our 2010 and 2012 bridge loans, our equipment notes payable and our venture loans.

Other Income

Other income consists primarily of: (i) interest income earned on our cash and cash equivalents; and (ii) exchange rate gains and losses on transactions denominated in a currency other than our functional currency, the U.S. dollar. Other income has historically included one-time, non-operating transactions such as the receipt of a federal grant or investment tax credit.

Critical Accounting Policies and Significant Judgments and Estimates

Our management s discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or GAAP. The preparation of these financial statements requires us to make informed estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. These items are monitored and analyzed by us for changes in facts and circumstances, and material changes in these estimates could occur in the future. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ materially from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 1 to our audited consolidated financial statements appearing elsewhere in this prospectus, we believe that the following accounting policies are critical to the process of making significant judgments and estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

Revenue Recognition

Our revenues generally consist of upfront, milestone and collaborative research activity payments received under our collaboration agreements. Some of our agreements contain multiple elements, including technological and territorial licenses and research and development services. In accordance with these agreements, we may be eligible for upfront fees, collaborative research funding and milestones. Revenues are recognized when all four of the following criteria are met: (i) persuasive evidence that an arrangement exists; (ii) delivery of the products and/or services has occurred; (iii) the selling price is fixed or determinable; and (iv) collectability is reasonably assured. Additional information on each type of revenue is outlined below.

Collaboration Agreements entered into prior to 2011

For multiple-element agreements entered into prior to January 1, 2011 and not materially modified thereafter, such as our agreements with Astellas, Teva and Genoptix, we analyzed the agreement to determine whether the elements within the agreement could be separated or whether they must be accounted for as a single unit of accounting. If the delivered element, which for us is commonly a license, has stand-alone value and the fair value of the undelivered elements, which for us are generally collaborative research activities, can be determined, we recognized revenue

separately under the residual method as the elements under the agreement are delivered. If the delivered element does not have stand-alone value or if the fair value of the undelivered element cannot be determined, the agreement is then accounted for as a single unit of accounting, with consideration received under the agreement recognized as revenue on the straight-line basis over the estimated period of performance, which for us is generally the expected term of the research and development plan.

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Collaboration Agreements entered into or materially modified after December 31, 2010

We apply an accounting standard which addresses the determination of the unit(s) of accounting for multiple-element arrangements and how the arrangement s consideration should be allocated to each unit of accounting. We adopted this new accounting standard on a prospective basis for all multiple-element arrangements entered into on or after January 1, 2011 and for any multiple-element arrangements that were entered into prior to January 1, 2011 but materially modified on or after January 1, 2011. We have not entered into nor materially modified any agreements since December 31, 2010.

Each required deliverable in a collaboration agreement is evaluated to determine if it qualifies as a separate unit of accounting. For us, this determination is generally based on whether the deliverable has stand-alone value to the customer. The arrangement s consideration is then allocated to each separate unit of accounting based on the relative selling price of each deliverable. The estimated selling price of each deliverable is determined using the following hierarchy of values: (i) vendor-specific objective evidence of fair value; (ii) third-party evidence of selling price; and (iii) best estimate of selling price, or BESP. The BESP reflects our best estimate of what the selling price would be if the deliverable was regularly sold by us on a stand-alone basis. We expect, in general, to use the BESP for allocating consideration to each deliverable. In general, the consideration allocated to each unit of accounting is then recognized as the related goods or services are delivered limited to the consideration that is not contingent upon future deliverables.

Milestones

Revenue is recognized when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved, provided that the milestone event is substantive. A milestone event is considered to be substantive if its achievability was not reasonably assured at the inception of the arrangement and our efforts led to the achievement of the milestone (or if the milestone was due upon the occurrence of a specific outcome resulting from our performance). Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty s performance are not considered to be milestone events. If both of these criteria are not met, the milestone payment is recognized over the remaining minimum period of our performance obligations under the arrangement, if any. We assess whether a milestone is substantive at the inception of each arrangement.

Generally, the milestone events contained in our collaborative agreements coincide with the progression of the drug candidates from clinical trial to regulatory approval and then to commercialization. The process of guiding a clinical trial candidate through clinical trials, having it approved and ultimately commercialized is highly uncertain. As such, the milestone payments we may earn from our partners involve a significant degree of risk to achieve. Therefore, as a drug candidate progresses through the stages of its life-cycle, the value of the drug candidate generally increases.

Other

Collaboration agreements also include potential payments for product royalties and sharing of operating profits. To date, we have not received payments or recorded any revenue from any of these other sources.

Clinical Trial Accruals

As part of the process of preparing its financial statement, we are required to estimate our expenses resulting from our obligations under contracts with vendors and consultants and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations which vary from contract to contract,

and may result in payment flows that do not match the periods over which materials or services are provided to us under such contracts. Our objective is to reflect the appropriate trial expenses in our financial statements by matching those expenses with the period in which the services and efforts are expended. We account for these expenses according to the progress of the trial as measured by patient progression and the

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timing of various aspects of the trial. We determine accrual estimates through financial models, taking into account discussion with applicable personnel and outside service providers as to the progress or state of consummation of trials, or the services completed. During the course of a clinical trial, we adjust our rate of clinical expense recognition if actual results differ from its estimates. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on the facts and circumstances known us at that time. 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 our reporting amounts that are too high or too low for any particular period. Through December 31, 2013, there have been no material adjustments to our prior period estimates of accrued expenses for clinical trials. Our clinical trial accrual is dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors.

Stock-Based Compensation

We grant options to purchase our common stock to our employees and directors under our equity incentive plan. Eligible employees can also purchase share of our common stock under our employee stock purchase plan at the lower of: (i) 85% of the fair market value on the first day of a six-month purchase and offering period; or (ii) 85% of the fair market value on the last date of the six-month offering period. In addition, we grant options to purchase our common stock to non-employees under our equity incentive plan.

We account for stock-based compensation by measuring and recognizing compensation expense for all stock-based payments made to employees and non-employee directors based on estimated grant date fair values. We use the straight-line method to allocate compensation cost to reporting periods over each optionee s requisite service period, which is generally the vesting period. We estimate the fair value of our stock-based awards to employees and non-employee directors using the Black-Scholes option pricing model. The Black-Scholes model requires the input of subjective assumptions, including the risk-free interest rate, expected volatility, expected term and the fair value of the underlying common stock on the date of grant, among other inputs.

Risk-free Interest Rate. The risk-free interest rate assumption is based on zero-coupon U.S. Treasury instruments that have terms consistent with the expected term of our stock option grants.

Expected Dividend Yield. We have never declared or paid any cash dividends and do not presently plan to pay cash dividends in the foreseeable future.

Expected Volatility. Due to our limited operating history and lack of Company-specific historical and implied volatility, the expected volatility rate used to value stock option grants is estimated based on volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the pharmaceutical and biopharmaceutical industry in a similar stage of development.

Expected Term. We elected to utilize the simplified method for plain vanilla options to estimate the expected term of stock option grants. Under this approach, the weighted-average expected term is presumed to be the average of the vesting term and the contractual term of the option.

In August 2011, our board of directors authorized the repricing of the exercise price of 1,291 options previously granted to employees, consultants and directors. We analyzed the fair value of the options immediately before and after the repricing and determined that the incremental value of the repricing was immaterial, as the repriced options were granted at an exercise price above the fair market value of our common stock.

For 2013, 2012 and 2011, we have reduced stock-based compensation expense recognized in the Statement of Operations to reflect for estimated forfeitures. Forfeitures are estimated at the time of grants and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

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Warrant Liabilities

Prior to our IPO, we issued freestanding warrants to purchase shares of our redeemable convertible preferred stock and common stock. The redeemable convertible preferred stock warrants were exercisable for shares of Series C, Series D and Series D-2 redeemable convertible preferred stock and were classified as liabilities in the accompanying consolidated balance sheets, as the terms for redemption of the underlying security were outside our control. The warrants are recorded at fair value using either the Black-Scholes option pricing model, probability weighted expected return model or a binomial model. We used the Black-Scholes option pricing model to value all warrants except the warrants issued in connection with our venture loans in March 2010 and the warrants issued in conjunction with the sale of Series D-2 preferred stock in May 2011. A binomial model was used to value the March 2010 warrants, as these warrants included anti-dilution terms that could change the settlement amount. The final share amounts and exercise price of these warrants became fixed upon the closing of our Series D-2 financing in May 2012 and the warrants have been valued using the Black-Scholes option pricing model thereafter. The grant date fair value of the Series D-2 warrants issued in May 2011 was determined as part of a probability weighted expected return model since the warrants contained provisions whereby the ultimate number of warrants that would become exercisable was based on operational milestones included in the warrants. Subsequent to our determination in 2011 that the warrants would become fully exercisable, we began to value the warrants using the Black-Scholes option pricing model. Upon our IPO, all classes of preferred stock were converted into common stock. Upon this conversion, the preferred stock warrants were classified as a component of stockholders equity and are no longer subject to remeasurement.

Our outstanding common stock warrants issued in connection with our Series E financing in 2012 continue to be classified as liabilities in the accompanying consolidated balance sheet as they contain provisions that could require us to settle the warrants in cash. The fair value of all of these common warrants is re-measured at each financial reporting date with any changes in fair value being recognized in change in fair value of warrant and derivative liabilities, a component of other income (expense), in the accompanying consolidated statements of operations. We will continue to re-measure the fair value of the warrant liabilities until the exercise or the expiration of the related warrant.

Redeemable Non-Controlling Interest

Prior to our IPO, the redeemable non-controlling interest in our subsidiary, Ambit Canada, was created through the issuance of redeemable convertible preferred stock put obligations, or the puts, which have elements similar to a liability instrument and are classified as liabilities in the accompanying consolidated balance sheets at fair value. At each reporting period prior to the IPO, we adjusted the carrying value of the redeemable non-controlling interest by the net loss attributable to the redeemable non-controlling interest. Any difference between the fair value and the adjusted carrying value of the redeemable non-controlling interest was recorded as an adjustment to additional paid-in capital and presented as a component of net loss attributable to common stockholders in the accompanying consolidated statements of operations. Upon the IPO, the Class C, Series D or Series D-2 shares of Ambit Canada are held by GrowthWorks Canadian Fund Ltd., or GrowthWorks were put to Ambit in exchange for common stock in Ambit. Subsequent to this transaction, no Class C, Series D or Series D-2 shares of Ambit Canada were held by GrowthWorks Canadian Fund Ltd., or GrowthWorks, or any other third party, at which time the redeemable non-controlling interest was reclassified to additional paid-in capital.

Net Operating Loss and Research and Development Tax Credit Carryforwards

As of December 31, 2013, we had federal and California tax net operating loss carryforwards of $192.8 million and $181.7 million, respectively, which begin to expire in 2022 and 2016, respectively, unless previously utilized. As of December 31, 2013, we also had federal and California research and development tax credit carryforwards of $6.4 million and $6.1 million, respectively. The federal research and development tax credit carryforwards will begin to

expire in 2024. The California research and development tax credit carryforwards are available indefinitely.

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Utilization of the net operating losses and credits may be subject to a substantial annual limitation due to ownership change limitations provided by the Internal Revenue Code of 1986, as amended. The annual limitation may result in the expiration of our net operating losses and credits before we can use them. We have recorded a valuation allowance on all of our deferred tax assets, including our deferred tax assets related to our net operating loss and research and development tax credit carryforwards.

JOBS Act

In April 2012, the JumpStart Our Business Startups Act of 2012, or the JOBS Act, was signed into law. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an emerging growth company. As an emerging growth company, we are electing not to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision not to take advantage of the extended transition period is irrevocable. In addition, we are in the process of evaluating the benefits of relying on the other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, if as an emerging growth company we choose to rely on such exemptions, we may not be required to, among other things, (i) provide an auditor s attestation report on our system of internal controls over financial reporting pursuant to Section 404, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) 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 (auditor discussion and analysis), and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer s compensation to median employee compensation. These exemptions will apply for a period of five years following the completion of our initial public offering or until we no longer meet the requirements of being an emerging growth company, whichever is earlier.

The above listing is not intended to be a comprehensive list of all of our accounting policies. In many cases, the accounting treatment of a particular transaction is specifically dictated by GAAP. There are also areas in which our management s judgment in selecting any available alternative would not produce a materially different result. Please see our audited consolidated financial statements and notes thereto included elsewhere in this prospectus, which contain accounting policies and other disclosures required by GAAP.

Results of Operations

Comparison of the Years Ended December 31, 2013 and 2012

Collaboration Agreement Revenues. We recorded revenues of $27.1 million and $17.6 million for the years ended December 31, 2013 and 2012, respectively, primarily under our agreement with Astellas. The increase of approximately $9.5 million was primarily due to an increase in license fee amortization revenue being recognized during 2013. In March 2013, we received a notice of termination of the agreement from Astellas, which termination was effective in September 2013. Accordingly, the remaining deferred revenue related to the upfront license fee was recognized over the period from March 2013 through September 2013. This increase was partially offset by a reduction in cost reimbursement revenue due to lower quizartinib research and development expenses and the termination of the collaboration agreement with Astellas in early September 2013. The reduction in quizartinib research and development expenses was due to a reduction in the number of patients being treated and followed in our Phase 2 clinical trial, in which enrollment was completed in late 2011.

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Research and Development Expenses. Our research and development expenses were $26.3 million and $36.7 million for the years ended December 31, 2013 and 2012, respectively. A comparison of research and development expenses by category is as follows (in thousands):

	Years Ended December 31,		Increase/ (Decrease)
	2013	2012	
Outside services	$ 17,310	$ 27,287	$ (9,977)
Salaries and personnel	6,953	6,304	649
Facilities and operations	2,021	3,140	(1,119)
Total	$ 26,284	$ 36,731	$ (10,447)

Outside Services. Expenses for outside services, such as for CROs and investigator sites, decreased approximately $10.0 million from $27.3 million for the year ended December 31, 2012 to $17.3 million for the year ended December 31, 2013. The decrease was due to lower quizartinib research and development expenses, resulting from a reduction in the number of patients being treated and followed in the Phase 2 clinical trial. The reduction was also due in part to a reduction in pre-clinical activities.

Salaries and Personnel. Expenses for salaries and personnel increased approximately $649,000 from $6.3 million for the year ended December 31, 2012 to $7.0 million for the year ended December 31, 2013. The increase was primarily due to increased stock-based compensation expense in 2013 as compared to 2012. The increase was also due to an increase in personnel-related costs in 2013 as compared to 2012. As of December 31, 2013, we employed 30 full-time employees in research and development, an increase of three full-time employees from December 31, 2012.

Facilities and Operations. Expenses for facilities and operations decreased approximately $1.1 million from $3.1 million for the year ended December 31, 2012 to $2.0 million for the year ended December 31, 2013. The decrease was primarily due to lower monthly rent expense associated with Ambit s new facility as compared to Ambit s Sorrento Valley Boulevard facility. In addition, costs associated with the facility other than rent, such as property taxes and utilities, decreased between the Sorrento Valley Boulevard facility and the new facility. Ambit moved into the new facility in March 2013.

General and Administrative Expense. General and administrative expenses increased approximately $3.8 million from $6.6 million for the year ended December 31, 2012 to $10.3 million for the year ended December 31, 2013. The increase was primarily due to increases in stock-based compensation, personnel-related costs, legal and accounting expenses, insurance costs, and investor relation costs, the majority of which are due to our becoming a public company in 2013. As of December 31, 2013, we employed 19 full-time employees in general and administrative functions, an increase of six full-time employees.

Interest Expense. Interest expense decreased approximately $1.4 million from $1.7 million for the year ended December 31, 2012 to $323,000 for the year ended December 31, 2013. The decrease in interest expense was primarily due to a decrease of approximately $814,000 in cash interest expense related to venture loans as we paid down more principal near the maturity of the loans in September 2013. These loans were paid in full in September 2013. In addition, the overall decrease was due to a $597,000 decrease in 2012 non-cash interest associated with the 2012 bridge loans, as these loans were converted into shares of Series E preferred stock in October 2012.

Change in Fair Value of Warrant and Derivative Liabilities. During the year ended December 31, 2013, the $4.1 million change in fair value of the warrant liabilities primarily related to an increase in the fair value of the underlying

common stock, which increased $3.64 from $6.00 at December 31, 2012 to $9.64 at December 31, 2013. During the year ended December 31, 2012, the $2.3 million change in fair value of the warrant liabilities primarily related to an increase in the fair value of the underlying preferred securities. Although the various estimated enterprise values utilized in our probability-weighted valuation models did not change significantly, the timing and probabilities changed as we progressed toward our IPO.

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Comparison of the Years Ended December 31, 2012 and 2011

Collaboration Agreement Revenues. We recorded revenues of $17.6 million and $23.8 million for the years ended December 31, 2012 and 2011, respectively, under our agreement with Astellas. The decrease of approximately $6.2 million was primarily due to a reduction in reimbursement from Astellas due to lower quizartinib research and development expenses. The reduction in quizartinib research and development expenses was due to a reduction in the number of patients being treated and followed in our Phase 2 clinical trial for quizartinib.

Research and Development Expenses. Our research and development expenses were $36.7 million and $50.7 million for the years ended December 31, 2012 and 2011, respectively. A comparison of research and development expenses by category is as follows (in thousands):

	Years Ended December 31,		
	2012	2011	Decrease
Outside services	$ 27,287	$ 36,334	$ (9,047)
Salaries and personnel	6,304	8,836	(2,532)
Facilities and operations	3,140	5,535	(2,395)
Total	$ 36,731	$ 50,705	$ (13,974)

Outside Services. Expenses for outside services, such as for CROs and investigator sites, decreased approximately $9.0 million from $36.3 million for the year ended December 31, 2011 to $27.3 million for the year ended December 31, 2012. The decrease was due to lower quizartinib research and development expenses, resulting from a reduction in the number of patients being treated and followed in the Phase 2 clinical trial. The reduction was also due in part to a reduction in pre-clinical activities and lower CMC development costs.

Salaries and Personnel. Expenses for salaries and personnel decreased approximately $2.5 million from $8.8 million for the year ended December 31, 2011 to $6.3 million for the year ended December 31, 2012. The decrease was primarily due to reductions in headcount which reflected our focus on cost reduction in light of uncertainty in the public and private financial markets. As of December 31, 2012, we employed 27 full-time employees in research and development.

Facilities and Operations. Expenses for facilities and operations decreased approximately $2.4 million from $5.5 million for the year ended December 31, 2011 to $3.1 million for the year ended December 31, 2012. The decrease was due to the accrual of an early termination fee in July 2011 upon our exercise of the early termination provision of our lease agreement and to a reduction in monthly rent expense for our Sorrento Valley Boulevard facility.

General and Administrative Expense. General and administrative expenses decreased approximately $2.3 million from $8.9 million for the year ended December 31, 2011 to $6.6 million for the year ended December 31, 2012. The decrease was primarily due to decreases in severance, legal and accounting costs and stock-based compensation expense. The decrease in severance costs was primarily due to reductions in headcount which reflected our focus on cost reduction in light of uncertainty in the public and private financial markets. The decrease in stock-based compensation expense was due to a combination of headcount turnover resulting in cancellation of options, coupled with a decline in our common stock value. As of December 31, 2012, we employed 13 full-time employees in general and administrative.

Interest Expense. Interest expense decreased approximately $2.8 million from $4.5 million for the year ended December 31, 2011 to $1.7 million for the year ended December 31, 2012. The decrease in interest expense was primarily due to the $2.1 million decrease in 2012 non-cash interest associated with the 2012 bridge loans compared to the 2011 non-cash interest associated with the 2010 bridge loans. In addition, there was a $528,000 decrease in cash interest expense related to venture loans as we pay down more principal near the maturity of the loans.

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Other Income. Other income decreased approximately $1.5 million from $1.5 million for the year ended December 31, 2011 to $29,000 for the year ended December 31, 2012. The decrease was primarily due to our earning a credit of $1.3 million in 2011 under the 2010 Canadian Scientific Research and Experimental Development, or SR&ED, Tax Incentive Program. The SR&ED program provides certain Canadian controlled companies with a refundable investment tax credit for a portion of qualified research and experimental expenditures. We were not eligible for a similar credit in 2012.

Change in Fair Value of Warrant and Derivative Liabilities. During the year ended December 31, 2011, we recorded a $2.4 million loss and a $1.6 million gain, respectively, from the change in the fair value of redeemable convertible preferred stock warrant liabilities and derivative liability conversion feature. The change in fair value of the redeemable convertible preferred stock warrant liabilities in 2011 primarily related to an increase in the aggregate estimated value of the Series D-2 financing warrants as a result of delays in the clinical development process. Upon our conclusion in September 2011 that the Series D-2 financing warrants would become fully exercisable as a result of missing operational milestones, we changed from a probability-based model to a Black-Scholes option pricing model under which the full 26.6 million Series D-2 financing warrants were valued at current fair value. This increase in fair value related to the increase in expected warrant shares and was offset by declines in the estimated fair value of our Series D-2 redeemable convertible preferred stock. At the time of conversion, a final mark-to-market adjustment was recorded on our derivative liability conversion feature, resulting in a $1.6 million gain. We determined the derivative had zero value at conversion since the effective conversion price of the related bridge loans was less than the fair value of the underlying preferred stock at the conversion date.

During the year ended December 31, 2012, the $2.3 million change in fair value of the warrant liabilities primarily related to an increase in the fair value of the underlying preferred securities. Although the various estimated enterprise values utilized in our probability-weighted valuation models did not change significantly, the timing and probabilities changed as we progressed toward an IPO.

Liquidity and Capital Resources

We have incurred losses since inception and negative cash flows from operating activities for 2013, 2012 and 2011. As of December 31, 2013, we had an accumulated deficit of $248.2 million. We anticipate that we will continue to incur net losses for the foreseeable future as we: (i) continue the development and potential commercialization of our lead drug candidate, quizartinib, (ii) continue our research and development programs to advance our internal product pipeline and (iii) incur additional costs associated with being a public company.

On March 31, 2010, we received $12.0 million in gross proceeds from the issuance of two secured promissory notes under the Venture Loan and Security Agreement with Compass Horizon Funding Company LLC and Oxford Finance Corporation, or the Venture Loans. The Venture Loans were designated for general working capital and to repay $2.2 million of prior working capital notes. The annual interest rate, excluding the final payment, is fixed at 12.25%. The final payment, which was due October 1, 2013, included additional interest of 3.0% of the initial loan amount, or $360,000, which was accreted over the life of the note using the effective interest method and is included in interest expense. In accordance with the terms of the Venture Loans, we made payments of interest only during the initial 12 month period May 1, 2010 through April 1, 2011 and commenced making principal and interest payments May 1, 2011 for the remaining 30 months. The Venture Loans are secured by a first priority security interest in all assets, excluding intellectual property, for which we have provided a negative pledge. The notes were paid in full as of September 30, 2013.

In May 2012, we entered into a series of agreements, pursuant to which certain investors loaned us a total of $11.5 million, or the 2012 Bridge Financing. Outstanding balances under the 2012 Bridge Financing accrue interest at a rate

of 10% per annum. In October 2012, these notes, including accrued interest on the notes, were converted into 17,008,346 shares of our Series E redeemable convertible preferred stock in connection with the closing of the Series E financing. In conjunction with our IPO, all shares of our preferred stock were converted into common stock.

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In May 2012, we, Ambit Canada and GrowthWorks entered into a Note Purchase Agreement, or the 2012 Canadian Agreement, pursuant to which GrowthWorks loaned Ambit Canada $1.5 million, or the 2012 Canadian Bridge Financing. Outstanding balances under the 2012 Canadian Bridge Financing accrue interest at a rate of 10% per annum. The 2012 Canadian Convertible Promissory Notes are generally convertible on the same terms as the 2012 Convertible Promissory Notes, but for shares of Ambit Canada. In October 2012, these notes plus accrued interest were converted into 2,247,223 Class E non-voting shares of Ambit Canada in connection with the Series E financing. In conjunction with our IPO, all shares of our preferred stock were converted into common stock.

In May 2013, we completed our IPO pursuant to a registration statement on Form S-1 that was declared effective on May 15, 2013. In the IPO, we sold 8,125,000 shares of our common stock at a price of $8.00 per share. As a result of the IPO, we raised a total of $58.1 million in net proceeds after deducting underwriting discounts and commissions of approximately $4.6 million and offering expenses of approximately $2.3 million. Concurrent with the closing of the IPO, we sold 3,134,495 shares of common stock to certain of our existing shareholders in a concurrent private placement at the IPO price of $8.00 per share and received net proceeds of $25.1 million.

From our inception through December 31, 2013, we have funded our consolidated operations primarily through the placements of equity and convertible debt securities and upfront payments and milestones from our collaboration agreements. Additionally, we have funded a portion of our operations from service revenues and additional funding under our collaboration agreements. As of December 31, 2013, we had cash and cash equivalents of approximately $71.2 million.

The following table sets forth a summary of the net cash flow activity for each of the periods set forth below (in thousands):

	Years Ended December 31,		
	2013	2012	2011
Net cash used in operating activities	$ (27,302)	$ (28,772)	$ (39,626)
Net cash (used in) provided by investing activities	(631)	46	(153)
Net cash provided by financing activities	82,016	29,759	18,594
Effect of exchange rate changes on cash	(375)	31	(246)
Net increase (decrease) in cash and cash equivalents	$ 53,708	$ 1,064	$ (21,431)

Cash used in operating activities decreased $1.5 million from $28.8 million for the year ended December 31, 2012 to $27.3 million for the year ended December 31, 2013. This decrease was driven by a decrease in our net loss of $15.8 million from $27.0 million for the year ended December 31, 2012 to $11.3 million for the year ended December 31, 2013. The decrease was partially offset by increased utilization of deferred revenues (a use of cash) from $6.4 million in 2012 to $20.7 million in 2013. Both the decrease in the net loss and the offsetting increase in utilization of deferred revenues were due to the termination of the Astellas agreement, which ended in September 2013. Changes in working capital and deferrals other than deferred revenues in the years ended December 31, 2013 and 2012 used cash of $2.2 million and provided cash of $2.6 million, respectively. Non-cash expenses increased approximately $2.3 million from $4.5 million for the year ended December 31, 2012 to $6.8 million for the year ended December 31, 2013.

Cash used in operating activities decreased $10.9 million from $39.6 million for the year ended December 31, 2011 to $28.8 million for the year ended December 31, 2012. This decrease was driven by a decrease in our net loss of $10.4 million from $37.4 million for the year ended December 31, 2011 to $27.0 million for the year ended December 31, 2012. Changes in working capital and deferrals in the years ended December 31, 2011 and 2012 used cash of $8.6

million and $6.3 million, respectively. Non-cash expenses decreased approximately $1.9 million from $6.4 million for the year ended December 31, 2011 to $4.5 million for the year ended December 31, 2012.

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During the year ended December 31, 2013, investing activities used cash of $631,000, primarily due to purchases of property and equipment, partially offset by proceeds from the sale of property and equipment. During the year ended December 31, 2012, investing activities provided cash of $46,000, primarily due to proceeds from the sale of property and equipment. During the year ended December 31, 2011, investing activities used cash of $153,000, primarily due to purchases of property and equipment, partially offset by proceeds associated with the sale of our kinase profiling services business in October 2010.

Financing activities provided cash of $82.0 million, $29.8 million and $18.6 million for the years ended December 31, 2013, 2012 and 2011, respectively. During the year ended December 31, 2013, our IPO and concurrent private offering raised net proceeds of approximately $83.5 million. In addition, sales of put shares to GrowthWorks during the first quarter of 2013 raised approximately $2.7 million. During the year ended December 31, 2012, we issued convertible notes of approximately $13.0 million. Additionally, we issued redeemable convertible preferred stock, net of issuance costs, which provided approximately $22.0 million. During the year ended December 31, 2011, we issued redeemable convertible preferred stock, net of issuance costs, of approximately $19.1 million. We also issued Series D-2 put shares, which provided approximately $2.6 million.

Principal debt payments were $4.4 million, $4.8 million and $3.1 million for the years ended December 31, 2013, 2012 and 2011, respectively. Principal payments on our venture loan commenced in April 2011, were made throughout 2012 and ended in September 2013, due to the loan reaching its contractual conclusion.

The financial statements of our Canadian subsidiary are measured using the local currency as the functional currency. The effect of exchange rate on cash relates to the fluctuation in exchange rate of the Canadian dollar to the U.S. dollar.

Operating Capital Requirements

Contractual Obligations. Under our collaboration agreement with Astellas, through the effective date of the termination, we shared equally with Astellas all agreed-upon development costs related to quizartinib in the United States and European Union, and research costs on other compounds under the agreement.

Our most significant clinical trial expenditures are to CROs. The contracts with CROs generally are cancellable, with notice, at our option and do not have any cancellation penalties. These items are not included in the table below.

The following table summarizes our contractual obligations at December 31, 2013 (in thousands):

		Payments Due by Period			
	Total	Less than 1 Year	1-3 Years	3-5 Years	More than 5 Years
Operating lease obligations	$ 3,361	$ 739	$ 1,417	$ 1,205	$
Total	$ 3,361	$ 739	$ 1,417	$ 1,205	$

Our commitments for operating leases relate to our lease of office and laboratory space in San Diego, California.

Our future capital requirements are difficult to forecast and will depend on many factors, including:

the progress, costs and results of our Phase 2b clinical trial, our anticipated Phase 3 clinical trial and future trials that may be required to support regulatory approval and label expansion for quizartinib;

the outcome, timing and cost of regulatory approvals;

the initiation, progress, timing and results of preclinical studies and clinical trials for any of our other drug candidates;

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the costs and timing of establishing sales, marketing and distribution capabilities;

delays that may be caused by changing regulatory requirements;

the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims; and

the extent to which we acquire or invest in businesses, products or technologies.

We believe our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations through at least the next 12 months. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements (as defined by applicable SEC regulations) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

Our cash and cash equivalents as of December 31, 2013 consisted of cash and money market funds. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition and/or results of operation.

Foreign Currency Risk

Our balance sheet as of December 31, 2013 includes cash and cash equivalent balances of $5.0 million denominated in Canadian dollars through our Canadian subsidiary, Ambit Canada. The majority of Ambit Canada s operational activities are denominated in Canadian dollars. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate losses during the years ended December 31, 2013, 2012 and 2011.

Effects of Inflation

We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

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Item 8. Financial Statements and Supplementary Data

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of

Ambit Biosciences Corporation

We have audited the accompanying consolidated balance sheets of Ambit Biosciences Corporation as of December 31, 2013 and 2012, and the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders equity (deficit), and cash flows for each of the three years in the period ended December 31, 2013. 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 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. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly we express no such opinion. An audit also 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.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Ambit Biosciences Corporation at December 31, 2013 and 2012, and the consolidated results of its operations and comprehensive loss and its cash flows for each of the three years in the period ended December 31, 2013, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

San Diego, California

March 20, 2014

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Ambit Biosciences Corporation

CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share data)

	December 31, 2013	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$ 71,189	$ 17,481
Accounts receivable	1,000	
Prepaid expenses and other current assets	911	1,231
Total current assets	73,100	18,712
Property and equipment, net	785	560
Deposits and other assets		717
Restricted cash	63	
Total assets	$ 73,948	$ 19,989
Liabilities, convertible preferred stock and stockholders equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 4,711	$ 7,290
Accrued payroll and related expenses	1,997	1,313
Current portion of notes payable, net of discount		4,320
Current portion of deferred revenue		6,362
Warrant liabilities	9,650	10,540
Total current liabilities	16,358	29,825
Deferred revenue, net of current portion		14,309
Redeemable non-controlling interest		3,323
Commitments and contingencies		
Convertible preferred stock, $0.001 par value:		
Authorized shares 10,000,000 and 170,990,763 at December 31, 2013 and December 31, 2012		
Redeemable convertible preferred stock:		
Issued and outstanding shares 0 and 121,826,424 at December 31, 2013 and December 31, 2012, respectively; liquidation preference $0 and $202,475 at December 31, 2013 and December 31, 2012, respectively		157,076
Convertible preferred stock;		
Issued and outstanding shares 0 and 1,590,014 at December 31, 2013 and December 31, 2012, respectively; liquidation preference $0 and $13,702 at December 31, 2013 and December 31, 2012, respectively		13,702
Stockholders equity (deficit):		
Common stock, $0.001 par value;		

Authorized shares 200,000,000 and 225,000,000 at December 31, 2013 and December 31, 2012, respectively; issued and outstanding shares 17,919,031 and 3,990 at December 31, 2013 and December 31, 2012, respectively	18	
Additional paid-in capital	306,064	38,678
Accumulated other comprehensive (loss) income	(326)	47
Accumulated deficit	(248,166)	(236,971)
Total stockholders equity (deficit)	57,590	(198,246)
Total liabilities, convertible preferred stock and stockholders equity (deficit)	$ 73,948	$ 19,989

See accompanying notes to these financial statements.

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Ambit Biosciences Corporation

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share data)

	Year Ended December 31,		
	2013	**2012**	**2011**
Revenues:			
Collaboration agreements	$ 27,093	$ 17,633	$ 23,843
Operating expenses:			
Research and development	26,284	36,731	50,705
General and administrative	10,342	6,550	8,905
Gain on sale of kinase profiling services business	(2,500)		
Noncash gain on sale of kinase profiling services business		(2,497)	(2,108)
Total operating expenses	34,126	40,784	57,502
Loss from operations	(7,033)	(23,151)	(33,659)
Other income (expense):			
Interest expense	(323)	(1,737)	(4,502)
Other income (expense)	143	29	1,538
Change in fair value of warrant and derivative liabilities	(4,072)	(2,291)	(795)
Total other income (expense)	(4,252)	(3,999)	(3,759)
Loss before income taxes	(11,285)	(27,150)	(37,418)
Benefit for income taxes	(29)	(121)	
Consolidated net loss	(11,256)	(27,029)	(37,418)
Net loss/(income) attributable to redeemable non-controlling interest	61	382	(213)
Net loss attributable to Ambit Biosciences Corporation	(11,195)	(26,647)	(37,631)
Other comprehensive income/(loss):			
Foreign currency translation	(373)	28	(242)
Comprehensive loss	$ (11,629)	$ (27,001)	$ (37,660)
Net loss per share attributable to common stockholders, basicand diluted	$ (1.19)	$ (16,591.99)	$ (25,886.60)
Weighted average shares outstanding, basic and diluted	11,024,175	1,848	1,358

See accompanying notes to these financial statements.

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Ambit Biosciences Corporation

CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS EQUITY (DEFICIT)

(in thousands, except share data)

	Redeemable Convertible Preferred Stock		Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Deficit
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2010	20,861,279	$ 97,256	1,595,794	$ 13,752	1,316	$	$ 28,998	$ 261	$ (172,693)	$ (143,434)
Issuance of common stock upon exercise of stock options					54		79			79
Cash paid for Series D-2 shares	27,762,411	17,328								
Issuance of Series D-2 redeemable convertible preferred stock upon conversion of bridge loans	27,123,172	16,138								
Series D-2 issuance costs		(382)								
Accretion to redemption value of redeemable convertible preferred stock		2,000					(2,000)			(2,000)
Change in fair value of redeemable non-controlling interest							4,477			4,477
Net income attributable to redeemable									(213)	(213)

non-controlling interest									
Stock-based compensation						1,387			1,387
Foreign currency translation							(242)		(242)
Consolidated net loss								(37,418)	(37,418)
Balance at December 31, 2011	75,746,862	132,340	1,595,794	13,752	1,370	32,941	19	(210,324)	(177,364)
Cancellation of Series D-2 warrants						2,851			2,851
Cash paid for Series E shares	31,906,341	22,334							
Issuance of Series E redeemable convertible preferred stock upon conversion of bridge loans	17,008,346	11,906							
Series E issuance costs		(374)							
Cash paid for common stock					1,437	3			3
Conversion of preferred stock to common stock	(2,835,125)	(5,910)	(5,780)	(50)	1,183	5,960			5,960
Warrant exchange		(197)				197			197
Issuance of common stock warrants in connection with Series E financing		(6,184)							
Accretion to redemption value of redeemable convertible preferred stock		3,161				(3,161)			(3,161)
Change in fair value of redeemable						(854)			(854)

non-controlling interest				
Net loss attributable to redeemable non-controlling interest			382	382
Stock-based compensation	741			741
Foreign currency translation		28		28
Consolidated net loss			(27,029)	(27,029)

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Ambit Biosciences Corporation

CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS EQUITY (DEFICIT) (Continued)

(in thousands, except share data)

	Redeemable Convertible Preferred Stock		Convertible Preferred Stock		Common Stock			Accumulated Other		
	Shares	Amount	Shares	Amount	Shares	Amount	Additional Paid-In Capital	Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Deficit
lance at cember 31, 12	121,826,424	157,076	1,590,014	13,702	3,990		38,678	47	(236,971)	(198,24
cretion to emption ue of eemable nvertible ferred stock		3,634					(3,634)			(3,63
ange in fair ue of eemable n-controlling erest							1,747			1,74
t loss ributable to eemable n-controlling erest									61	6
nversion of ferred rrant liability equity							4,689			4,68
nversion of eemable n-controlling erest to mmon stock					530,092	1	4,240			4,24
nversion of ferred stock common ck	(121,826,424)	(160,710)	(1,590,014)	(13,702)	5,918,981	6	174,405			174,41
uance of mmon stock					8,125,000	8	58,101			58,10

on initial blic offering, of offering ts							
uance of mmon stock on private cement ering		3,134,495	3	25,072			25,07
uance of mmon stock on exercise warrants		177,573		273			27
uance of mmon stock on exercise stock options		10,064		60			6
uance of mmon stock ough ployee stock n purchase		18,836		128			12
ck-based mpensation				2,305			2,30
reign rency nslation					(373)		(37
nsolidated loss						(11,256)	(11,25
lance at cember 31, 13	$	$ 17,919,031	$ 18	$ 306,064	$ (326)	$ (248,166)	$ 57,59

See accompanying notes to these financial statements.

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Ambit Biosciences Corporation

CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Year Ended December 31,		
	2013	**2012**	**2011**
Operating activities			
Consolidated net loss	$ (11,256)	$ (27,029)	$ (37,418)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation expense	377	903	1,069
Change in fair value of redeemable convertible preferred stock warrant and derivative liabilities	4,072	2,291	795
Noncash interest expense	117	803	3,140
Bad debt expense			2
Stock-based compensation expense	2,305	741	1,387
Loss (gain) on disposal of property and equipment	(34)	(197)	27
Deferred revenue	(20,671)	(6,379)	(6,362)
Noncash gain on sale of kinase profiling services business		(2,497)	(2,108)
Changes in operating assets and liabilities:			
Accounts receivable	(1,000)	3,510	643
Prepaid expenses and other current assets	320	(34)	159
Accounts payable and accrued expenses	(2,216)	110	(1,412)
Accrued payroll and related expenses	684	(994)	452
Net cash used in operating activities	(27,302)	(28,772)	(39,626)
Investing activities			
Proceeds from sale of kinase profiling services business			400
Proceeds from sale of property and equipment	45		
Purchase of property and equipment	(613)	46	(553)
Restricted cash	(63)		
Net cash (used in) provided by investing activities	(631)	46	(153)
Financing activities			
Proceeds from issuance of common stock and exercise of stock options	188	3	79
Proceeds from issuance of common stock, net of offering costs	83,515	(331)	
Net proceeds from issuance of redeemable convertible preferred stock		21,960	19,052
Proceeds from issuance of put shares	2,725	(44)	2,566
Proceeds from notes payable		13,000	
Payments on notes payable	(4,412)	(4,829)	(3,103)
Net cash provided by financing activities	82,016	29,759	18,594
Effect of exchange rate changes on cash	(375)	31	(246)

Net change in cash and cash equivalents	53,708	1,064	(21,431)
Cash and cash equivalents at beginning of the period	17,481	16,417	37,848
Cash and cash equivalents at end of the period	$ 71,189	$ 17,481	$ 16,417
Supplemental schedule of noncash investing and financing activities			
Issuance of bridge notes and accrued interest into redeemable preferred stock	$	$ 11,906	$ 16,138
Issuance of Series D-2 warrants in connection with Series D-2 financing	$	$	$ 2,106
Contributed capital related to cancelled Series D-2 financing warrants	$	$ 2,851	$
Issuance of common warrants in connection with Series E financing	$	$ 6,184	$
Conversion of redeemable non-controlling interest to common stock	$ 4,241	$	$
Conversion of preferred warrant liability to equity	$ 4,689	$	$
Conversion of preferred stock to common stock	$ 174,409	$	$
Supplemental disclosures of cash flow information			
Interest paid	$ 252	$ 982	$ 1,392
Taxes paid	$ 1	$	$ 20

See accompanying notes to these financial statements.

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Ambit Biosciences Corporation

Notes to Consolidated Financial Statements

1. Organization and Summary of Significant Accounting Policies

Ambit Biosciences Corporation (Ambit or the Company), formerly Aventa Biosciences Corporation, was incorporated in Delaware on May 17, 2000 and is located in San Diego, California. Ambit is a biopharmaceutical company focused on the discovery, development and commercialization of drugs to treat unmet medical needs in oncology, autoimmune and inflammatory diseases by inhibiting kinases that are important drivers for those diseases.

Initial Public Offering and Concurrent Private Placement

The Company closed its initial public offering (IPO) in May 2013, selling 8,125,000 shares of common stock at a price of $8.00 per share, resulting in gross proceeds of approximately $65.0 million and net proceeds of approximately $58.1 million, after underwriting and other expenses of approximately $6.9 million (consisting of $4.6 million in underwriting discounts and commissions and $2.3 million in other offering expenses). In connection with the completion of the IPO, all outstanding convertible preferred stock converted into 6,449,073 shares of common stock.

Concurrent with the IPO, the Company sold 3,134,495 shares of common stock to certain of the Company s existing stockholders in a concurrent private placement at the IPO price of $8.00 per share and received net proceeds of approximately $25.1 million.

Effective upon the closing of the IPO, 1,845,329 shares of common stock were reserved for future issuance under the Company s 2013 Equity Incentive Plan, including 1,214,212 shares of common stock reserved for issuance upon the exercise of outstanding options issued under the Company s 2011 Amended and Restated Equity Incentive Plan and 6,117 shares of common stock previously reserved for issuance under the Company s 2011 Amended and Restated Equity Incentive Plan, in each case that were added to the shares reserved under the 2013 Equity Incentive Plan upon its effectiveness.

Effective upon the closing of the Company s IPO, 125,000 shares of common stock were reserved for future issuance under the Company s 2013 Employee Stock Purchase Plan.

Principles of Consolidation

The consolidated financial statements include the accounts of the Company, its wholly-owned subsidiary Ambit Europe Limited (Ambit Europe) and its controlled subsidiary, Ambit Biosciences (Canada) Corporation (Ambit Canada), which became a wholly-owned subsidiary upon the Company s IPO. All intercompany transactions and balances among the consolidated entities are eliminated in consolidation. Ambit Europe was incorporated in England in June 2008. As of December 31, 2013, there have been no significant transactions related to Ambit Europe. Ambit Canada was formed in Canada in December 2004.

Reverse Stock Splits

On October 26, 2012 and April 24, 2013, the Company filed amended and restated certificates of incorporation under which each share of the Company s common stock was split on a 1-for-100 basis and a 1-for-24 basis, respectively. The accompanying consolidated financial statements and notes to the consolidated financial statements give retroactive effect to the reverse splits for all periods presented.

Foreign Currency Translation and Transactions

The accompanying consolidated financial statements are presented in U.S. dollars. The financial statements of Ambit Canada are measured using the local currency as the functional currency. The translation of Ambit

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Canada s assets and liabilities to U.S. dollars is made at the exchange rate in effect at the balance sheet date, while the financing related accounts are translated at the rate in effect at the date of the underlying transaction. Equity accounts, including retained earnings, are translated at historical rates. The translation of the statement of comprehensive income (loss) data is made at the average rate in effect for the period. The translation of operating cash flow data is made at the average rate in effect for the period, and investing and financing cash flow data is translated at the rate in effect at the date of the underlying transaction. Translation gains and losses are recognized within accumulated other comprehensive income (loss) in the accompanying consolidated balance sheets. Transactions expected to be settled in a currency other than the functional currency are remeasured to current exchange rates each period until such transaction is settled. The resulting gain or loss is included in other income (expense) in the accompanying consolidated statements of operations and comprehensive loss. There were no material transaction gains or losses during any period presented in the accompanying financial statements.

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make informed estimates and assumptions that impact the amounts reported in the consolidated financial statements and accompanying notes. The most significant estimates in the Company s consolidated financial statements relate to the fair value of the common and preferred stock warrant liabilities, redeemable non-controlling interest, derivative liability-conversion feature, clinical trial accruals and stock options. In addition, there is a significant amount of judgment used in the area of revenue recognition. Actual results could differ materially from those estimates and assumptions.

Cash and Cash Equivalents

Cash and cash equivalents consist of cash and highly liquid investments, which include money market funds that are readily convertible into cash without prior notice or penalty. The Company considers securities with remaining maturities of three months or less, at the date of purchase, to be cash equivalents. Cash and cash equivalents are recorded at face value or cost, which approximates fair market value.

Concentration of Credit Risk and Significant Customers

Financial instruments, which potentially subject the Company to significant concentration of credit risk, consist primarily of cash, cash equivalents and accounts receivable. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits and management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

Restricted Cash

In connection with the Company s lease, the Company issued a letter of credit in the amount of approximately $63,000. The letter of credit is renewable annually for the term of the lease with the landlord and is collateralized by cash held in an interest-bearing account at a bank. The security deposit balance is shown as restricted cash on the accompanying consolidated balance sheets.

Property and Equipment

Property and equipment are recorded at cost and depreciated using the straight-line method over the estimated useful lives of the assets (generally three to five years). Leasehold improvements are stated at cost and depreciated on a straight-line basis over the lesser of the remaining term of the related lease or the estimated useful lives of the assets.

Construction in progress is not depreciated until the underlying asset is placed in service. Repairs and maintenance costs are charged to expense as incurred.

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Impairment of Long-Lived Assets

Long-lived assets consist primarily of property and equipment. An impairment loss is recorded if and when events and circumstances indicate that assets might be impaired and the undiscounted cash flows estimated to be generated by those assets are less than the carrying amount of those assets. While the Company s current and historical operating losses and negative cash flows are indicators of impairment, management believes that future cash flows to be received support the carrying value of its long-lived assets and, accordingly, has not recognized any impairment losses during the years ended December 31, 2013, 2012 and 2011.

Fair Value of Financial Instruments

The carrying amounts of accounts receivable, accounts payable and accrued liabilities are considered to be representative of their respective fair values because of the short-term nature of those instruments.The carrying amount of the warrant liabilities and redeemable non-controlling interest represent their fair values.

Warrant Liabilities

Prior to the Company s IPO, redeemable convertible preferred stock warrants exercisable for shares of Series C, Series D and Series D-2 redeemable convertible preferred stock were classified as liabilities in the accompanying consolidated balance sheets, as the terms for redemption of the underlying security were outside the Company s control. The Company s outstanding common stock warrants issued in connection with its Series E financing in 2012 are classified as liabilities in the accompanying consolidated balance sheets as they contain provisions that could require the Company to settle the warrants in cash. The warrants were recorded at fair value using either the Black-Scholes option pricing model, probability weighted expected return model or a binomial model, depending on the characteristics of the warrants. The fair value of these warrants is re-measured at each financial reporting period with any changes in fair value being recognized as a component of other income (expense) in the accompanying consolidated statements of operations and comprehensive loss.

Upon the closing of the IPO and the conversion of the of the underlying preferred stock to common stock, the Company s warrants to purchase shares of Series C, Series D, and Series D-2 redeemable convertible preferred stock were converted into warrants to purchase shares of the Company s common stock. The aggregate fair value of these warrants upon the closing of the IPO was $4.7 million, which was reclassified from liabilities to additional paid-in capital in the accompanying consolidated balance sheets.

Revenue Recognition

The Company generates and recognizes revenue from collaboration agreements. Some of the Company s agreements contain multiple elements, including technological and territorial licenses and research and development services. In accordance with these agreements, the Company may be eligible for upfront fees, collaborative research funding and milestones. Revenues are recognized when all four of the following criteria are met: (i) persuasive evidence that an arrangement exists; (ii) delivery of the products and/or services has occurred; (iii) the selling price is fixed or determinable; and (iv) collectability is reasonably assured. Additional information on each type of revenue is outlined below.

Collaboration agreements entered into prior to 2011

For multiple-element agreements entered into prior to January 1, 2011 and not materially modified thereafter, such as the Company s agreement with Astellas Pharma Inc., the Company analyzed the agreement to determine whether the

elements within the agreement could be separated or whether they must be accounted for as a single unit of accounting. If the delivered element, which for the Company is commonly a license, had stand-alone value and the fair value of the undelivered elements, which for the Company was generally collaborative research activities, could be determined, the Company recognized revenue separately under the residual method as the elements under the agreement were delivered. If the delivered element did not have stand-alone value or if

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the fair value of the undelivered element could not be determined, the agreement was then accounted for as a single unit of accounting, with consideration received under the agreement recognized as revenue on the straight-line basis over the estimated period of performance, which for the Company was generally the expected term of the research and development plan.

Milestones

Revenue from milestones is recognized when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved, provided that the milestone event is substantive. A milestone event is considered to be substantive if its achievability was not reasonably assured at the inception of the arrangement and the Company s efforts led to the achievement of the milestone (or if the milestone was due upon the occurrence of a specific outcome resulting from the Company s performance). Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty s performance are not considered to be milestone events. If both of these criteria are not met, the milestone payment is recognized over the remaining minimum period of the Company s performance obligations under the arrangement. The Company assesses whether a milestone is substantive at the inception of each arrangement.

Generally, the milestone events contained in the Company s collaboration agreements coincide with the progression of the drug candidates from clinical trial, to regulatory approval and then to commercialization. The process of guiding a clinical trial candidate through clinical trials, having it approved and ultimately commercialized is highly uncertain. As such, the milestone payments the Company may earn from its partners involve a significant degree of risk to achieve. Therefore, as a drug candidate progresses through the stages of its life-cycle, the value of the drug candidate generally increases.

Collaboration agreements entered into or materially modified after December 31, 2010

In October 2009, the Financial Accounting Standards Board (FASB) issued a new accounting standard which amends the guidance on accounting for arrangements involving the delivery of more than one element. This standard addresses the determination of the unit(s) of accounting for multiple-element arrangements and how the arrangement s consideration should be allocated to each unit of accounting. The Company has not entered into nor materially modified any agreements since December 31, 2010.

Each required deliverable in a collaboration agreement is evaluated to determine if it qualifies as a separate unit of accounting. For the Company this determination is generally based on whether the deliverable has stand-alone value to the customer. The arrangement s consideration is then allocated to each separate unit of accounting based on the relative selling price of each deliverable. The estimated selling price of each deliverable is determined using the following hierarchy of values: (i) vendor-specific objective evidence of fair value; (ii) third-party evidence of selling price; and (iii) best estimate of selling price (BESP). The BESP reflects the Company s best estimate of what the selling price would be if the deliverable was regularly sold by the Company on a stand-alone basis. The Company expects, in general, to use the BESP for allocating consideration to each deliverable. In general, the consideration allocated to each unit of accounting is then recognized as the related goods or services are delivered limited to the consideration that is not contingent upon future deliverables.

The Company has recognized the following revenue from collaboration agreements:

Year Ended December 31,

	2013	2012	2011
		(in thousands)	
Upfront licensing fees	$ 20,671	$ 6,379	$ 6,362
Collaborative research activities	5,422	11,254	17,481
Milestones	1,000		
Total revenue from collaborative arrangements	$ 27,093	$ 17,633	$ 23,843

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Deferred Revenue

Amounts received prior to satisfying the above revenue recognition criteria are recorded as deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized within the next 12 months are classified as non-current deferred revenue.

Research and Development

Research and development expenses include expenses incurred under agreements with contract research organizations, investigative sites and consultants that conduct the Company s clinical trials, employee-related expenses (such as salaries and benefits and stock-based compensation), costs of developing and acquiring clinical trial materials, facilities-related costs, and costs associated with other research activities and regulatory approvals. Research and development costs are expensed as incurred. Prepaid clinical expenses and advance payments for goods and services that will be used in future research and development activities are included in prepaid expenses and other current assets in the consolidated balance sheets. Prepaid clinical expenses were $405,000 and $861,000 as of December 31, 2013 and 2012, respectively.

Clinical Trial Accruals

The Company is required to estimate its expenses resulting from its obligations under contracts with vendors and consultants and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations which vary from contract to contract, and may result in payment flows that do not match the periods over which materials or services are provided to the Company under such contracts. The Company s objective is to reflect the appropriate trial expenses in its financial statements by matching those expenses with the period in which the services and efforts are expended. The Company accounts for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. The Company determines accrual estimates through financial models, taking into account discussion with applicable personnel and outside service providers as to the progress or state of consummation of trials, or the services completed. During the course of a clinical trial, the Company adjusts its rate of clinical expense recognition if actual results differ from its estimates. The Company makes estimates of its accrued expenses as of each balance sheet date in its financial statements based on the facts and circumstances known to the Company at that time. Although the Company does not expect its estimates to be materially different from amounts actually incurred, its 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 the Company reporting amounts that are too high or too low for any particular period. Through December 31, 2013, there have been no material adjustments to the Company s prior period estimates of accrued expenses for clinical trials. The Company s clinical trial accrual is dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors.

Patent Expenses

Costs related to filing and pursuing patent applications are recorded as general and administrative expense as incurred since recoverability of such expenditures is uncertain.

Comprehensive Loss

Comprehensive loss encompasses the change in equity during a period from transactions and other events and circumstances from non-owner sources. The Company s only component of other comprehensive loss is the foreign currency translation adjustments related to Ambit Canada. Comprehensive loss has been reflected in the statements of

operations and comprehensive loss and as a separate component of the statements of stockholders equity (deficit) for all periods presented.

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Stock-Based Compensation

Stock-based compensation expense represents the cost of the grant date fair value of employee and non-employee director stock option grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable or the performance condition has been achieved. The weighted-average estimated fair value of employee and non-employee director stock options granted (other than through a repricing) during the years ended December 31, 2013, 2012 and 2011 were $7.80 per share, $3.71 per share and $323.85 per share, respectively, using the Black-Scholes option pricing model with the following weighted-average assumptions (annualized percentages):

	Years Ended December 31,		
	2013	**2012**	**2011**
Risk-free interest rate	1.9%	0.9%	1.2%
Expected dividend yield			
Expected volatility	68.8%	67.4%	63.1%
Expected term (in years)	6.0	6.0	6.1

The risk-free interest rate is based on United States Treasury zero-coupon bonds with maturities similar to those of the expected term of the award being valued. The expected dividend yield is based on the Company s history and expectation in the foreseeable future of not paying dividends. Due to the limited historical data of the Company s fair value, the estimated volatility incorporates the historical volatility of comparable companies whose shares are publicly available covering a timeframe similar to that of the expected term. The expected term of the award is calculated using the simplified method because of the lack of relevant historical data.

As stock-based compensation expense recognized in the statement of operations and comprehensive loss for the years ended December 31, 2013, 2012 and 2011 is based on awards ultimately expected to vest, it should be reduced for estimated forfeitures. Forfeitures are estimated at the time of grants and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

Income Taxes

The Company accounts for income taxes under 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 included in the financial statements. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date.

The Company recognizes net deferred tax assets to the extent that the Company believes these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

The Company records uncertain tax positions on the basis of a two-step process whereby (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50 percent likely to be realized upon ultimate settlement with the related tax authority. The Company recognizes interest and penalties related to unrecognized tax benefits within income tax expense. Any accrued interest and penalties are included within the related tax liability line.

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Segments

The Company operates in one business segment. The Company reports segment data based on the management approach. The management approach designates the internal reporting that is used by management for making operating and investment decisions and evaluating performance as the source of its reportable segments.

Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents are comprised of convertible preferred stock, redeemable convertible preferred stock puts (non-controlling interest), warrants for the purchase of convertible preferred and common stock, convertible notes payable and options outstanding under the Company s stock option plan. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to the Company s net loss position.

The computation for basic and diluted EPS was as follows (in thousands, except share and per share data):

	Years ended December 31,		
	2013	2012	2011
Numerator for basic and diluted loss per share:			
Loss attributable to Ambit Biosciences Corporation	$ (11,195)	$ (26,647)	$ (37,631)
Accretion to redemption value of redeemable convertible preferred stock	(3,634)	(3,161)	(2,000)
Change in fair value of redeemable non-controlling interest	1,747	(854)	4,477
Net loss available to common stockholders	$ (13,082)	$ (30,662)	$ (35,154)
Denominator for basic and diluted loss per share:			
Weighted-average shares outstanding, basic and diluted	11,024,175	1,848	1,358
Basic and diluted net loss per share	$ (1.19)	$ (16,591.99)	$ (25,886.60)

Potentially dilutive securities not included in the calculation of diluted net loss per share attributable to common stockholders because to do so would be anti-dilutive are as follows (in common stock equivalent shares):

	Years ended December 31,		
	2013	2012	2011
Convertible preferred stock outstanding		5,918,981	4,014,981
Redeemable non-controlling interest		366,899	273,264
Warrants for convertible preferred stock		645,598	1,208,677
Warrants for common stock	1,621,159	1,155,322	1,057
Common stock options	1,683,377	1,220,138	2,836

3,304,536 9,306,938 5,500,815

Adoption of New Accounting Standards

On January 1, 2013, the Company adopted the provisions of Accounting Standards Update (ASU) 2013-02, Comprehensive Income (Topic 220) Reporting of Amounts Reclassified Out of Accumulated Other

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Comprehensive Income. ASU 2013-02 amends recent guidance related to the reporting of comprehensive income to enhance the reporting of reclassifications out of accumulated other comprehensive income. The adoption of ASU 2013-02 did not have a significant impact on the Company s financial statements.

2. Ambit Canada

Ambit Canada was incorporated on December 29, 2004. Since its inception through the Company s IPO in May 2013, through a series of debt and equity financing transactions between the Company, GrowthWorks Canadian Fund Ltd. (GrowthWorks), a Canadian investor, and Ambit Canada, the Company acquired and held between 36% and 50% of Ambit Canada s total outstanding shares and at least 50% of the outstanding voting shares of Ambit Canada.

Prior to the IPO, GrowthWorks held Class C, Series D-1, Series D-2 and Class E shares of Ambit Canada. These shares were subject to put options whereby GrowthWorks could exchange its non-voting shares in Ambit Canada for shares of the Company s redeemable convertible preferred stock. Immediately prior to the IPO, GrowthWorks exercised their put options and exchanged their shares of Ambit Canada for 1,538,461 shares of the Company s Series C-2 redeemable convertible preferred stock, 612,649 shares of the Company s Series D redeemable convertible preferred stock, 3,666,169 shares of the Company s Series D-2 redeemable convertible preferred stock and 6,163,916 shares of the Company s Series E redeemable convertible preferred stock, all of which shares were converted to common stock of the Company upon the IPO.

The Company has determined that, for all periods prior to Ambit Canada becoming a fully-owned subsidiary, Ambit Canada was a variable interest entity and that the Company was the primary beneficiary of Ambit Canada based on the following factors:

- The Company has the power to direct the activities of Ambit Canada which would most significantly impact Ambit Canada s economic performance, as the Company provides business services to Ambit Canada and Ambit Canada s business operations are supervised by members of the Company s executive team.

- The Company s obligation to absorb losses and receive benefits from Ambit Canada could potentially be significant and are disproportional to voting rights given GrowthWorks put options in the Company.

The Company determined that the investment held by GrowthWorks in Ambit Canada should be classified as a redeemable non-controlling interest as the shares of Ambit Canada were not in-substance common stock. In-substance common stock is an investment in an entity that has risk and reward characteristics that are substantially similar to that entity s common stock. Due to the liability characteristics associated with the shares of Ambit Canada held by GrowthWorks, the Company concluded that the investor s shares were not substantially similar to common stock. The liability characteristics include the investor s put rights, which provide it with the ability to exchange their shares in Ambit Canada for redeemable convertible preferred stock of the Company. Upon exercise of the puts by GrowthWorks, the Company also had the ability to pay GrowthWorks cash rather than issuing stock in the Company.

The redeemable non-controlling interest was initially valued using the fair value of the Company s Series C-2, Series D, Series D-2 and Series E redeemable convertible preferred stock. At each reporting period, the Company adjusted the carrying value of the redeemable non-controlling interest by the net income (loss) attributable to the redeemable non-controlling interest. Any difference between the fair value and the adjusted carrying value of the redeemable non-controlling interest was recorded as an adjustment to additional paid-in capital and presented as a component of net loss attributable to common stockholders in the accompanying consolidated statements of operations and

comprehensive income (loss). The redeemable non-controlling interest was measured at fair value until the IPO, at which time no Class C, Series D-1, Series D-2 or Class E shares of Ambit Canada were held by GrowthWorks or any other third party. The redeemable non-controlling interest was reclassified to additional paid-in capital.

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Until the IPO of the Company in 2013 and during the years ended December 31, 2012 and 2011, the Company adjusted the loss attributable to common stockholders as a result of decreases (increases) in the fair value of the redeemable non-controlling interest of approximately $1.7 million, $(0.9 million) and $4.5 million, respectively. The decreases in fair value reduced the loss attributable to common stockholders and increases in fair value increased the loss attributable to common stockholders.

The carrying amount and classification of Ambit Canada s assets and liabilities that are included in the consolidated balance sheets are as follows:

	December 31, 2012 (in thousands
Cash and cash equivalents	$ 2,420
Total assets of Ambit Canada	$ 2,420
Accounts payable and accrued expenses	$ 67
Total liabilities of Ambit Canada	$ 67

Consolidation of Ambit Canada s results of operations included the following:

	Year Ended December 31, 2012	2011
	(in thousands)	
Research and development expense	$ (646)	$ (1,026)
General and administrative expense		(56)
Interest expense	(87)	(116)
Other income (primarily from SR&ED credit)	60	1,533
Net loss of Ambit Canada	$ (673)	$ 335

Prior to the Company s IPO (and Ambit Canada becoming a fully-owned subsidiary), the loss of Ambit Canada was allocated to the redeemable non-controlling interest based on the relative ownership of Ambit Canada. As of December 31, 2012, the redeemable non-controlling interest held 60% of the outstanding shares of Ambit Canada. The Canadian Scientific Research and Experimental Development (SR&ED) Tax Incentive Program provides certain Canadian controlled companies with a refundable investment tax credit for a portion of the qualified research and experimental expenditures.

Consolidation of Ambit Canada s cash flows included the following:

	Year Ended December 31,	
	2012	2011
	(in thousands)	
Cash provided by (used in) operating activities	$ (746)	$ 394
Cash (used in) provided by financing activities	1,473	(2,179)
Effect of exchange rate on cash	31	(246)
Increase (decrease) in cash and cash equivalents of Ambit Canada	$ 758	$ (2,031)

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3. Balance Sheet Details

Property and equipment, net

	December 31,	
	2013	2012
	(in thousands)	
Scientific equipment	$ 2,905	$ 2,951
Computer hardware and software	1,325	1,416
Furniture and fixtures	358	170
Leasehold improvements	38	1,284
Construction in progress and deposits		141
	4,626	5,962
Accumulated depreciation	(3,841)	(5,402)
Property and equipment, net	$ 785	$ 560

Accounts payable and accrued expenses

	December 31,	
	2013	2012
	(in thousands)	
Accounts payable	$ 1,516	$ 4,320
Accrued clinical trials	1,862	996
Accrued expenses	1,333	1,609
Other		365
Accounts payable and accrued expenses	$ 4,711	$ 7,290

4. Fair Value Measurements

The following tables present information about the Company s financial assets and financial liabilities measured at fair value on a recurring basis as of December 31, 2013 and 2012, and indicate the fair value hierarchy of the valuation techniques utilized by the Company to determine such fair value. Fair value measurements are based on a three-tier hierarchy that prioritizes the inputs used to measure fair value. In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access. The Company classifies money market funds and United States Treasuries as Level 1 assets.

Fair values determined by Level 2 inputs utilize inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, and inputs other than quoted prices that are observable for the asset or liability, such as interest rates and yield curves that are observable at commonly quoted intervals. The Company obtains the fair value of Level 2 financial instruments from a third-party professional pricing service using quoted market prices for

identical or comparable instruments. The Company s professional pricing service gathers market prices from a variety of industry standard data providers, security master files from large financial institutions and other third-party sources. The service uses these multiple prices as inputs into a distribution-curve based algorithm to determine a fair value. The Company then validates the quoted fair values provided by the professional pricing service by comparing the service s assessment of the fair values of the Company s Level 2 investment portfolio balance against the fair values of the Company s Level 2 investment portfolio balance provided by the Company s investment managers. The Company classifies United States government agency securities as Level 2 assets. There were no transfers between Level 1 and Level 2 during the years ended December 31, 2013 or 2012.

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Level 3 inputs are unobservable inputs for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability. Financial assets and liabilities that are measured or disclosed at fair value on a recurring basis, and are classified within the Level 3 designation include the preferred stock and common stock warrant liabilities, derivative liabilities and the redeemable non-controlling interest. None of the Company s non-financial assets and liabilities are recorded at fair value on a non-recurring basis.

In certain cases, the inputs used to measure fair value may fall into different levels of the fair value hierarchy. In such cases, the level in the fair value hierarchy within which the fair value measurement in its entirety falls has been determined based on the lowest level input that is significant to the fair value measurement in its entirety. The Company s assessment of the significance of a particular input to the fair value measurement in its entirety requires considerable judgment, and considers factors specific to the asset or liability.

The following table presents our fair value hierarchy for assets and liabilities measured at fair value on a recurring basis at December 31, 2013 and 2012 (in thousands):

	Fair Value as of December 31, 2013			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds	$ 66,323	$ 66,323	$	$
Liabilities:				
Common warrants	$ 9,650	$	$	$ 9,650

	Fair Value as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds	$ 14,979	$ 14,979	$	$
Liabilities:				
Common warrants	$ 6,182	$	$	$ 6,182
Preferred warrants	4,358			4,358
Redeemable non-controlling interest	3,323			3,323
Total liabilities	$ 13,863	$	$	$ 13,863

The preferred stock and common stock warrant liabilities are recorded at fair value using the Black-Scholes option pricing model and the redeemable non-controlling interest is recorded at fair value based on the fair value of the underlying redeemable convertible preferred stock.

The following weighted-average assumptions were used in determining the fair value of the outstanding preferred stock and common stock warrant liabilities valued using the Black-Scholes option pricing model as of December 31, 2013 and December 31, 2012:

	December 31,	
	2013	**2012**
Risk-free interest rate	2.6%	1.6%
Expected dividend yield		
Expected volatility	62.9%	63.1%
Expected term in years	8.8	9.2

Of the inputs used to value the outstanding common stock warrant liabilities at December 31, 2013, the most subjective input is the Company s estimate of expected volatility. If volatility were increased to 80%, the weighted average fair market value of the outstanding common stock warrants outstanding would increase $0.03, or .3%.

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Prior to the Company s IPO, the following fair values per share of the redeemable convertible preferred stock and common stock were used in determining the fair value of the outstanding redeemable convertible preferred stock and common stock warrant liabilities and the redeemable non-controlling interest as of December 31, 2012:

	December 31, 2012
Series C redeemable convertible preferred stock	$ 0.31
Series D redeemable convertible preferred stock	0.70
Series D-2 redeemable convertible preferred stock	0.31
Series E redeemable convertible preferred stock	0.57
Common stock	6.00

Prior to the Company s IPO, the fair value of the redeemable convertible preferred stock and common stock was determined using a probability weighted expected return model. The key inputs into the model included the probability and timing of expected liquidity event dates, discount rates and the selection of appropriate market comparable transactions and multiples to apply to the Company s various historical and forecasted operational metrics.

The following table is a reconciliation for all liabilities measured at fair value using Level 3 unobservable inputs:

	Common Warrant Liabilities	Preferred Warrant Liabilities	Redeemable Non-Controlling Interest
		(in thousands)	
Balance at December 31, 2011	$	$ 4,916	$ 1,322
Issuance of common warrants in connection with Series E financing	6,184		
Issuance of shares of redeemable non-controlling interest			1,529
Warrants contributed to paid-in-capital		(2,851)	
Change in fair value	(2)	2,293	854
Net loss attributable to redeemable non-controlling interest			(382)
Balance at December 31, 2012	$ 6,182	$ 4,358	$ 3,323
Issuance of shares of redeemable non-controlling interest			2,725
Change in fair value	3,741	331	(1,747)
Net loss attributable to redeemable non-controlling interest			(61)
Exercise of common warrant liabilities	(273)		
Reclassification to additional paid-in capital upon the closing of the Company s IPO		(4,689)	(4,240)
Balance at December 31, 2013	$ 9,650	$	$

5. Warrants and Warrant Liabilities

The Company s outstanding warrant liabilities consisted of the following:

Issue Date	Expiration Date	Series	December 31, 2013 Exercise Price per Share	Shares Issuable upon Exercise	Fair Value
			(in thousands except share and per share data)		
October 2012	October 2022	Common	0.24	1,017,227	$ 9,624
November 2012	October 2022	Common	0.24	2,787	26
				1,020,014	$ 9,650

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Issue Date	Expiration Date	Series	Exercise Price per Share	December 31, 2012 Shares Issuable upon Exercise	Fair Value
			(in thousands except share and per share data)		
Preferred Warrants:					
October 2005	October 2015	Series C	4.30	232,558	$ 1
October 2005	October 2013	Series C	4.30	8,795	
December 2005	December 2013	Series C	4.30	7,207	
July 2006	July 2014	Series C	4.30	10,930	
October 2006	October 2014	Series C	4.30	2,336	
December 2006	December 2014	Series C	4.30	1,706	
March 2007	March 2015	Series C	4.30	3,052	
June 2007	June 2017	Series C	4.30	2,410	
September 2007	September 2017	Series C	4.30	93,023	2
August 2008	August 2016	Series D	5.06	2,369	
March 2010	March 2020	Series D-2	0.70	2,057,142	315
May 2011	May 2021	Series D-2	0.001	13,070,398	4,040
				15,491,926	4,358
Common Warrants:					
October 2012	October 2022	Common	0.24	1,058,221	6,163
November 2012	October 2022	Common	0.24	3,324	19
				1,061,545	6,182
					$ 10,540

Prior to the Company s IPO, redeemable convertible preferred stock warrants exercisable for shares of Series C, Series D and Series D-2 redeemable convertible preferred stock were classified as liabilities in the accompanying consolidated balance sheets, as the terms for redemption of the underlying security were outside the Company s control. The Company s outstanding common stock warrants issued in connection with its Series E financing in 2012 are classified as liabilities in the accompanying consolidated balance sheets as they contain provisions that could require the Company to settle the warrants in cash. The warrants were recorded at fair value using either the Black-Scholes option pricing model, probability weighted expected return model or a binomial model, depending on the characteristics of the warrants. The fair value of these warrants is re-measured at each financial reporting period with any changes in fair value being recognized as a component of other income (expense) in the accompanying consolidated statements of operations and comprehensive loss.

Upon the closing of the IPO and the conversion of the of the underlying preferred stock to common stock, the Company s warrants to purchase shares of Series C, Series D, and Series D-2 redeemable convertible preferred stock were converted into warrants to purchase shares of the Company s common stock.

The following table summarizes the warrants outstanding for purchase of common stock as of December 31, 2013 (excluding the warrants above that require liability accounting):

Shares Issuable Upon Exercise	Exercise Price	Expiration Date
14,409	$ 103.20	July 2014 September 2017
218	54.99	August 2016
72,970	21.84	June 2019 July 2019
78	2,184.00	July 2019
85,714	16.80	March 2020
20,690	36.96	September 2020
39	3,696.00	September 2020
407,027	0.02	May 2021
601,145		

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6. Debt, Commitments and Contingencies

The following is a reconciliation of the carrying amount of the Company s debt instruments:

	December 31,	
	2013	2012
Venture loans	$	4,412
Total notes payable		4,412
Discount on notes payable		(92)
Total notes payable, net of debt discount		4,320
Current portion of notes payable		(4,412)
Current portion of debt discount		92
Current portion of notes payable, net of debt discount		(4,320)
Notes payable, net of current portion	$	$

Venture Loans

On March 31, 2010, the Company received $12.0 million in gross proceeds from the issuance of two secured promissory notes under a Venture Loan and Security Agreement with Compass Horizon Funding Company LLC and Oxford Finance Corporation (the Venture Loans). The Venture Loans were designated for general working capital and to repay $2.2 million of prior working capital notes. The annual interest rate, excluding the final payment, is fixed at 12.25%. The final payment, which was made in September 2013, included additional interest of 3.0% of the initial loan amount, or $360,000, which was accreted over the life of the notes using the effective interest method and is included in interest expense in the accompanying consolidated statements of operations and comprehensive loss. In accordance with the terms of the notes, the Company made payments of only interest during the initial 12 month period May 1, 2010 through April 1, 2011 and commenced making principal and interest payments May 1, 2011 for the remaining 30 months. The Venture Loans were secured by a first priority security interest in all assets, excluding intellectual property, for which the Company provided a negative pledge.

The Company issued the lenders warrants to purchase shares of the Company s redeemable convertible preferred stock expiring in March 2020. The warrants contain a net issuance provision such that the lenders may exchange the warrants for shares without the payment of any additional cash consideration. The initial fair value of the warrants of $0.7 million was determined using a binomial model using Level 3 inputs and is recorded as a discount to the principal balance. This discount is amortized using the effective interest method over the 42 month term of the Venture Loans and is included in interest expense in the accompanying consolidated statements of operations and comprehensive loss. The warrants are exercisable for the purchase of an aggregate of 85,714 shares of the Company s common stock at an exercise price of $16.80 per share.

Facility Leases

The Company previously leased its office space under a noncancelable operating lease that expired on July 31, 2012. In July 2012, the lease was amended to allow the Company to extend the lease period on a month-by-month basis for

approximately $55,000 per month through February 2013. The Company was obligated to pay for operating expenses and certain repairs during the remaining lease term.

The Company entered into a new facility lease that has an initial term of approximately 5.5 years and commenced in March 2013. The base rent specified by the new facility lease agreement is approximately $51,000 per month for the first twelve months, escalating 3.0% annually to approximately $57,000 per month for the final twelve months of the initial term. The lease will expire in September 2018. The Company has an option to extend the term of the lease for an additional five years. The lease is subject to additional charges for property management, common area maintenance and other costs.

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Rent expense was $575,000, $1.2 million and $2.4 million in the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, future annual minimum lease payments for the operating lease are as follows (in thousands):

2014	$ 739
2015	758
2016	659
2017	680
2018	525
Thereafter	
	$ 3,361

Supplier Agreement

In connection with the sale of the kinase profiling service segment to DiscoveRx on October 21, 2010, the Company was obligated to purchase from DiscoveRx a minimum of $625,000 of screening services during each quarter through December 31, 2012.

Employment Agreements

Certain employees have employment agreements that provide for severance compensation in the event of termination or a change in control. These agreements can provide for a severance payment of up to 12 months of base salary in effect at the time of termination and continued health benefits at the Company s cost for up to 12 months.

Litigation

From time to time, the Company may be involved in various lawsuits, legal proceedings, or claims that arise in the ordinary course of business. Management does not believe any legal proceedings or claims pending at December 31, 2013, will have, individually or in the aggregate, a material adverse effect on its business, liquidity, financial position or results of operations. Litigation, however, is subject to inherent uncertainties, and an adverse result in these or other matters may arise from time to time that may harm the Company s business.

7. Redeemable Convertible Preferred Stock, Convertible Preferred Stock and Stockholders Equity (Deficit)

Prior to the conversion in the initial public offering in May 2013, our authorized, issued and outstanding shares of stock were as follows as of December 31, 2012:

	Shares Authorized	Shares Outstanding	Liquidation Preference Per Share	Liquidation Preference (in thousands)

Redeemable convertible preferred stock:				
Series C	7,076,718	4,380,631	$ 4.30	$ 18,837
Series C-2	1,538,461		3.25	
Series D	16,336,563	15,409,400	5.06	77,972
Series D-2	73,900,825	53,121,706	0.70	37,185
Series E	70,000,000	48,914,687	1.40	68,481
Total	168,852,567	121,826,424		$ 202,475
Convertible preferred stock:				
Series A	162,519	46,666	$ 7.50	$ 351
Series B	1,975,677	1,543,348	8.65	13,351
Total	2,138,196	1,590,014		$ 13,702

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Series D-2/D-3 Financing

In May 2011, the Company entered into a Series D-2 and Series D-3 Preferred Stock and Warrant Purchase Agreement (the Series D-2/D-3 Agreement), pursuant to which the Company agreed to issue and sell, and certain investors agreed to purchase up to an aggregate of 37,857,845 shares of series D-2 redeemable convertible preferred stock and up to an aggregate of 5,047,717 shares of series D-3 redeemable convertible preferred stock. In connection with the first closing in May 2011, the Company issued and sold 27,762,411 shares of Series D-2 redeemable convertible preferred stock at $0.70 per share and received $19.4 million in gross proceeds and incurred $0.4 million of issuance costs. In addition, an aggregate of $15.0 million of principal and an aggregate of $1.1 million of accrued interest on certain 2010 bridge loans converted into 27,123,172 shares of the Company s series D-2 redeemable convertible preferred stock in conjunction with the financing. Pursuant to the terms of the notes, the conversion was based on $0.595 per share which reflected a 15% discount to the per share amount paid by investors of $0.70.

In addition, the Company issued warrants for the purchase an aggregate of 26,583,858 shares of series D-2 redeemable convertible preferred stock to U.S. investors under the Series D-2/D-3 Agreement and to GrowthWorks pursuant to a subscription agreement (the May 2011 Warrants). The May 2011 Warrants have an exercise price of $0.001 per share and expire no later than May 16, 2021. The May 2011 Warrants became exercisable upon the attainment or lack of attainment of certain operational milestones. The warrants were initially valued at $2.1 million using a probability weighted expected return model. As of December 31, 2011, the Company had missed various operational milestones and determined that the warrants would become fully exercisable.

The Series D-2/D-3 Agreement also provided for a second closing under which the Company would receive $7.1 million in gross proceeds that was cancelled in connection with the bridge financing in May 2012.

2012 Bridge Loans

In May 2012, the Company entered into a Note Purchase Agreement, pursuant to which certain investors loaned the Company $11.5 million (the 2012 Bridge Financing). Outstanding balances under the 2012 Bridge Financing accrued interest at a rate of 10% per annum. The Company issued to its 2012 Bridge Financing investors Secured Subordinated Convertible Promissory Notes (the 2012 Convertible Promissory Notes), under which all outstanding principal and interest amounts were due on the earlier of (i) April 30, 2015 and (ii) immediately prior to an acquisition or asset transfer. In connection with the Company s Series E financing in October 2012, the aggregate $11.9 million of then outstanding principal and accrued interest automatically converted into 17,008,346 shares of Series E convertible preferred stock.

In May 2012, the Company, Ambit Canada and GrowthWorks entered into a Note Purchase Agreement (the 2012 Canadian Agreement), pursuant to which GrowthWorks loaned Ambit Canada $1.5 million (the 2012 Canadian Bridge Financing). Outstanding balances under the 2012 Canadian Bridge Financing accrued interest at a rate of 10% per annum. Ambit Canada issued Secured Subordinated Convertible Notes (the 2012 Canadian Convertible Promissory Notes) to GrowthWorks, under which all outstanding principal and interest amounts were due on April 30, 2015. The 2012 Canadian Convertible Promissory Notes were generally convertible on the same terms as the 2012 Convertible Promissory Notes, but for shares of Ambit Canada. In connection with the Company s Series E financing in October 2012, the aggregate $1.6 million of then outstanding principal and accrued interest converted into 2,247,223 Class E non-voting shares of Ambit Canada. As a result of this transaction, the Company s ownership of Ambit Canada was reduced to 40% and it still maintained voting control.

In connection with the 2012 Bridge Financing each investor amended their May 2011 Warrants such that warrants to purchase an aggregate of up to 26,583,858 shares of series D-2 redeemable convertible preferred stock became

exercisable for up to 13,291,929 shares of series D-2 redeemable convertible preferred stock. The $2.8 million fair value of the warrants contributed back to the Company in May 2011 was reclassified from the redeemable convertible preferred stock warrant liabilities to additional paid-in capital on the accompanying consolidated balance sheets.

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Series E Financing

In October 2012, the Company entered into a Series E Preferred Stock, Common Stock and Warrant Purchase Agreement (the Series E Agreement), pursuant to which the Company agreed to issue and sell, and certain investors agreed to purchase shares of Series E redeemable convertible preferred stock, shares of common stock and warrants to purchase common stock. The Series E Agreement was scheduled to close in three tranches. The financing agreement contains provisions whereby certain existing investors that did not participate in the Series E financing at specified levels will automatically have all of their existing preferred stock converted into common stock and reverse split on a 1-for-2,400 basis or 1-for-120 basis, depending on when the failure to participate occurs.

On October 25, 2012, the Company sold 1,437 shares of common stock under the Series E Agreement for aggregate proceeds of $3,450.

The first tranche closing of the preferred stock took place on October 26, 2012 and resulted in the sale of 48,726,367 shares of Series E redeemable convertible preferred stock at $0.70 per share. The Company received cash of $22.2 million for the issuance of 31,718,021 shares of Series E redeemable convertible preferred stock and issued 17,008,346 shares of Series E redeemable convertible preferred stock in exchange for the conversion of $11.9 million of principal and accrued interest of 2012 Convertible Promissory Notes. In connection with the first tranche closing, Ambit Canada issued 2,247,223 shares of its Class E preferred stock in exchange for the conversion of $1.6 million of principal and accrued interest of 2012 Canadian Convertible Promissory Notes. In addition, the Company issued fully exercisable warrants to purchase an aggregate of 1,058,221 shares of common stock at an exercise price of $0.24 per share that expire on October 26, 2022. If, in the event of an acquisition or asset transfer in which these warrants are not assumed, the Company would be required to purchase the warrants from each holder at its then fair value determined using a Black-Scholes option pricing model. As a result of the cash settlement provisions in the warrants they are classified as liabilities in the accompanying consolidated balance sheets. The initial $6.2 million fair value of the warrants was determined using the Black-Scholes option pricing model and was recorded as the initial carrying value of the common stock warrant liability and a reduction to the initial carrying value of the Series E redeemable convertible preferred stock.

In connection with the Series E financing, the Company solicited certain investors participation by way of a rights offering. As a result of such rights offering, in November 2012, the Company sold an additional 188,320 shares of Series E preferred stock for aggregate gross proceeds of $132,000 and issued fully exercisable warrants to purchase an aggregate of 3,324 shares of common stock at an exercise price of $0.24 per share that expire on October 26, 2022. Also in connection with the Series E financing, in November 2012 and December 2012, warrants to purchase an aggregate of up to 729 shares of the Company s common stock were exchanged or became exchangeable for warrants to purchase an aggregate of up to 72,977 shares of the Company s common stock with an initial exercise price of $21.84 per share. These warrants terminate 10 years after the date that the applicable cancelled warrant was issued. In addition, warrants to purchase an aggregate of up to 206 shares of the Company s common stock were exchanged or became exchangeable for warrants to purchase an aggregate of up to 20,697 shares of the Company s common stock with an initial exercise price of $36.96 per share. These warrants terminate 10 years after the date that the applicable cancelled warrant was issued. The $197,107 fair value of the 93,675 common warrants exchanged in 2012 was determined using the Black-Scholes option pricing model and was recorded as additional paid-in capital and a reduction to the initial carrying value of the Series E redeemable convertible preferred stock.

As a result of automatic conversion provisions in the Company s certificate of incorporation that were triggered in connection with the Series E financing, certain non-participating stockholders had their outstanding shares of preferred stock converted to common stock on a 1-for-2,400 basis. In addition, certain non-participating stockholders had their outstanding Series D-2 warrants cancelled. The carrying value of the preferred shares and the fair value of

the warrants were reclassified to additional paid-in capital upon the conversion or cancellation of the related instrument, as applicable.

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Common Stock

As of December 31, 2013, there were 17,919,031 shares of common stock outstanding. Each share of common stock is entitled to one vote. The holders of the common stock are also entitled to receive dividends whenever funds are legally available and when declared by our Board of Directors. Following the IPO, the Company filed an amended and restated certificate of incorporation to authorize 200,000,000 shares of common stock.

Stock Options

In January 2001, the Company adopted the 2001 equity incentive plan (the 2001 Plan). The 2001 Plan provided for the grant of incentive and non-statutory stock options, stock bonuses and rights to purchase restricted common stock by employees, directors and consultants of the Company with up to a ten-year contractual term and various vesting periods as determined by the Company s compensation committee or board of directors. The 2001 Plan provided that incentive stock options will be granted only to employees at no less than fair value of the Company s common stock (no less than 85.0% of the fair value for non-statutory stock options), as determined by the Board of Directors at the date of grant.

During 2011, the stockholders and Board of Directors of the Company approved various resolutions regarding the 2001 Plan. These resolutions served to rename the 2001 Plan to 2011 Equity Incentive Plan (the 2011 Plan), extend the term of the 2011 Plan to 2021 and increase the number of shares of common stock authorized for issuance ultimately to 6,811 shares.

During 2012, the Board of Directors of the Company approved various resolutions increasing the number of shares authorized for issuance under the 2011 Plan by 1,213,669 to 1,220,480 shares.

During 2013, the Board of Directors of the Company adopted the 2013 equity incentive plan (the 2013 Plan). The 2013 Plan was approved by the Company stockholders in May 2013, and became effective upon the IPO in May 2013. Effective upon the closing of the IPO, 1,845,329 shares of common stock were reserved for future issuance under the Company s 2013 Equity Incentive Plan, including 1,214,212 shares of common stock reserved for issuance upon the exercise of outstanding options issued under the Company s 2011 Amended and Restated Equity Incentive Plan and 6,117 shares of common stock previously reserved for issuance under the Company s 2011 Amended and Restated Equity Incentive Plan, in each case that were added to the shares reserved under the 2013 Equity Incentive Plan upon its effectiveness.

During 2013, the Company and certain employees agreed to cancel and retire a total of 3,548 options with strike prices equal to or in excess of $600 per share. The Company recognized non-cash stock-based compensation expense of $310,000 related to these cancellations and retirements.

Options granted under the 2011 and 2013 Plans generally expire no later than 10 years from the date of grant. Options generally vest and become fully exercisable over a period of four years. The exercise price of options granted generally cannot be less 100% of the fair market value of the Company s common stock on the date of grant. The Company issues new shares upon the exercise of stock options.

The following table summarizes stock option activity under the Plan:

	Number of Options	Weighted-Average Exercise Price
Options outstanding, December 31, 2012	1,220,138	$ 9.15
Granted	518,313	$ 12.51
Exercised	(10,064)	$ 6.00
Cancelled	(45,010)	$ 69.53
Options outstanding, December 31, 2013	1,683,377	$ 8.59

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The following table summarizes information about the Company s stock option plan as of December 31, 2013:

	Number of options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
December 31, 2013:				
Options vested and expected to vest	1,432,466	$ 8.45	9.1	$ 3,916
Options exercisable	321,864	$ 8.55	8.8	$ 1,168

The following table summarizes information about stock options:

	Year Ended December 31,	
	2013	2012
Weighted-average remaining contractual term (years) of options outstanding	9.1	9.9
Aggregate intrinsic value of options outstanding (in thousands)	$ 4,410	$
Intrinsic value of options exercised (in thousands)	$ 66	$
Cash received upon exercise of stock options (in thousands)	$ 60	$

Due to the Company s full valuation allowance and net operating loss carryforwards, it did not realize tax benefit from option exercise or recognized a tax benefit in the accompanying consolidated statement of operations and comprehensive loss, during any period presented.

Total stock-based compensation was allocated as follows:

	Years Ended December 31,		
	2013	2012	2011
	(in thousands)		
Research and development	$ 644	$ 129	$ 224
General and administrative	1,661	612	1,163
	$ 2,305	$ 741	$ 1,387

As of December 31, 2013, total unrecognized stock-based compensation costs related to non-vested stock options was approximately $5.2 million and the weighted-average term over which it is expected to be recognized is approximately 3.1 years.

Employee Stock Purchase Plan

During 2013, the Company adopted the 2013 Employee Stock Purchase Plan (the ESPP), which allows all eligible employees to purchase shares of the Company s common stock at the lower of (a) 85% of the fair market value of a share of the Company s common stock on the first date of a six-month offering and purchase period or (b) 85% of the

fair market value of a shares of the Company s common stock on the date of purchase. Employees may authorize the Company to withhold up to 15% of their compensation during any purchase period, subject to certain limitations. The ESPP authorizes up to 125,000 shares to be granted. At December 31, 2013, 18,836 shares of common stock have been issued under the ESPP at an average price of $6.80 per share.

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Common Stock Reserved for Future Issuance

The Company had common shares reserved for future issuance upon the exercise or conversion of the following as of December 31, 2013 and 2012:

	December 31,	
	2013	2012
Redeemable convertible preferred stock and convertible preferred stock		5,918,981
Redeemable non-controlling interest		366,899
Warrants for redeemable convertible preferred stock and convertible preferred stock		645,598
Warrants for common stock	1,621,159	1,155,322
Common stock option grants issued and outstanding	1,683,377	1,220,138
Common shares available for grant under the stock option plan	148,340	191
Common shares reserved under the ESPP	106,164	
Total common shares reserved for future issuance	3,559,040	9,307,129

8. Collaboration Agreements

Astellas Pharma Inc. and Astellas US LLC

In December 2009, the Company entered into an agreement with Astellas Pharma Inc. and Astellas US LLC (collectively Astellas) to jointly, research, develop and commercialize certain FLT3 kinase inhibitors in oncology and non-oncology indications. Under the agreement, the Company granted Astellas an exclusive, worldwide license, with limited rights to sublicense, develop, commercialize and otherwise exploit quizartinib and certain metabolites and derivatives of those compounds. In addition, the agreement provides that the Company and Astellas will conduct a five-year joint research program related to preclinical development of certain designated follow-on compounds to quizartinib. Astellas has sole ownership of all regulatory materials and approvals related to the compounds in exchange for certain payments described below and their commitment to jointly develop, and then commercialize and promote, products based on the licensed technology.

In December 2009, as partial consideration for the license rights granted to Astellas, Astellas paid the Company an upfront, non-refundable fee of $40.0 million. It was determined that there is one unit of accounting under the Astellas contract. As a result, the $40.0 million non-refundable license fee was initially recognized on a straight-line basis over 6.25 years, which was the Company s estimate of the maximum period over which it will be jointly developing the lead product, quizartinib.

The Company records its research and development costs as incurred in the statement of operations and comprehensive loss and recognizes revenue from such collaborative research activities for 50% of the eligible costs. Any amounts due to Astellas for the Company s share of costs incurred by Astellas are presented as research and development costs.

On March 7, 2013 Astellas exercised the right to terminate the Company s agreement, effective September 2, 2013. Until September 2, 2013 Astellas and the Company continued to share agreed-upon development costs equally. Subsequent to September 2, 2013, the Company is solely responsible for development costs associated with quizartinib.

Astellas is obligated to reimburse the Company for half of certain agreed-upon costs in connection with the transition of Astellas development activities to the Company. These costs are substantially fully expensed by the Company as of the end of 2013.

The Company recorded revenues under this agreement of $26.1 million, $17.6 million and $23.8 million in the years ended December 31, 2013, 2012 and 2011, respectively. Deferred revenues under this agreement were $0 and $20.7 million as of December 31, 2013 and 2012, respectively.

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Teva Pharmaceutical Industries Ltd.

In November 2006, the Company entered in an exclusive collaboration agreement with Cephalon, Inc., aimed at identifying and developing clinical candidates that demonstrate activity towards the two designated target kinases of the collaboration: the BRAF kinase and a second kinase determined by a joint research committee. In October 2011, Teva Pharmaceutical Industries Ltd. (Teva) acquired Cephalon, Inc. Under the agreement, both parties contributed certain intellectual property to the collaboration and agreed to a period of exclusivity during which neither party would engage in any research related to a collaboration target compound with a third-party. Teva is solely responsible for worldwide clinical development and commercialization of collaboration compounds, subject to the Company s option, exercisable during certain periods following completion of the first proof-of-concept study in humans and only with the consent of Teva, to co-develop and co-promote CEP-32496.

Cephalon, Inc. paid the Company an upfront fee of $15.5 million as partial consideration for access to the Company s profiling technology and the licenses the Company contributed to the collaboration. The upfront fee was recognized over the collaborative period of the agreement. The collaborative portion of the agreement ended in November 2009, at which point the Company had completed all its research obligations under the agreement.

The Company assessed the event-based payments specified by the agreement in accordance with the guidance and determined that all event-based payments were earned based on Teva s performance. As such, these payments do not meet the definition of a milestone in accordance with the guidance. The Company s obligations under the agreement came to an end in 2009. In 2013, Teva notified the Company that due to Teva s progress, the Company earned a $1.0 million event-based payment.

The Company recorded $1.0 million and $0 in revenues under this agreement in the years ended December 31, 2013 and 2012. There were no deferred revenues under this agreement as of December 31, 2013 or 2012.

The Company may receive tiered royalty payments ranging from the mid-single digits to the low double digits calculated as a percentage of net sales of the collaboration compounds, including CEP-32496, subject to certain offsets. Royalties are payable to the Company on a product-by-product, country-by-country basis beginning on the date of first commercial sale in a country and ending on the later of 10 years after the date of such sale in that country or the expiration date of the last to expire patent covering the licensed product in that country. The agreement remains in effect on a product-by-product, country-by-country basis until all royalty obligations expire. Both parties have a right to terminate the agreement early if the other party enters bankruptcy or upon an uncured breach by the other party. Teva may also terminate the agreement in its discretion upon 90 days written notice to the Company, or if available cash falls below a certain threshold.

Genoptix Medical Laboratory

In September 2010, the Company entered into a collaboration agreement with Genoptix Medical Laboratory, a Novartis company (Genoptix), to develop a laboratory diagnostic test to identify patients that harbor ITD mutations in their FLT3 receptor tyrosine kinase. Under this agreement, Genoptix will contribute its expertise in developing laboratory tests and the Company will supply certain patient samples to the collaboration. Genoptix has the right to commercialize the approved test. The Company has initially paid for the development activities under the collaboration pursuant to an agreed-upon budget and expenses such development costs as incurred. The Company is entitled to single-digit tiered royalty payments from Genoptix until the Company has recouped the development costs plus an additional predetermined percentage of such costs. To date, the Company has not recognized any revenues under this agreement.

The Company and Genoptix may assign the agreement to a third party in connection with the transfer or sale of all or substantially all of the business to which the agreement relates, whether by merger, sale of stock, sale of assets or otherwise, provided that in the event of such a transaction with a third party, intellectual property rights

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of such third party will not be included in the intellectual property rights licensed under the agreement with Genoptix to the extent such intellectual property rights would not have been licensed under the agreement in the absence of such transaction.

The agreement with Genoptix expires when the last payment obligation of either party under the agreement is fulfilled. Both parties have a right to terminate the agreement early upon an uncured material breach by the other party. Genoptix may terminate the agreement upon 45 days notice for an unresolved dispute between the parties regarding the development of the laboratory diagnostic test, upon 30 days notice if there is an unresolved dispute regarding the Company s payment of specified development costs and upon written notice if Ambit, its affiliates, or its sublicensees of certain intellectual property where Ambit does not, within ten days of receipt of notice from Genoptix, terminate such sublicense, contest or assist other parties in contesting Genoptix s rights regarding such intellectual property. The Company may terminate the agreement upon 60 days notice for any reason subject to payment by the Company of any outstanding development costs, and immediately if Genoptix or a party providing services to Genoptix relating to the development of the laboratory diagnostic test is debarred under the provisions of the Generic Drug Enforcement Act of 1992.

Bristol-Myers Squibb Company

In October 2007, the Company and Bristol-Myers Squibb Company (BMS) entered into a license agreement for the worldwide development and commercialization of AC480. Under the agreement, the Company acquired an exclusive, worldwide, non-transferable license to exploit certain patents and other intellectual property related to AC480. The Company also maintained limited rights to sublicense AC480, subject to a right of first offer retained by BMS. In August 2012, the Company terminated the license agreement and relinquished all rights associated with the agreement.

9. Income Taxes

Income (loss) before income taxes is as follows:

	Years Ended December 31,		
	2013	2012	2011
	(in thousands)		
United States operations	$ (11,261)	$ (26,477)	$ (37,753)
Foreign operations	(24)	(673)	335
	$ (11,285)	$ (27,150)	$ (37,418)

The provision for (benefit) from taxes consists of the following:

	Years Ended December 31,		
	2013	2012	2011
	(in thousands)		
Current:			
Federal	$ (30)	$ (122)	$

State and local	1	1	
Foreign			
Total current	(29)	(121)	
Total deferred			
Total provision	$ (29)	$ (121)	$

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A reconciliation between the Company s effective tax rate and the federal statutory tax rate is as follows:

	Years Ended December 31,		
	2013	2012	2011
Income tax benefit at federal statutory rate	(35.0)%	(35.0)%	(35.0)%
Income tax benefit at state statutory rate	(5.7)%	(5.6)%	(5.8)%
Research and development credits	(11.3)%	(0.7)%	(1.8)%
Change in valuation allowance	25.2%	36.1%	40.6%
Accretion	14.7%	3.4%	0.9%
Equity compensation	5.5%	0.8%	1.4%
Other, net	6.4%	0.5%	(0.3)%
	(0.2)%	(0.5)%	0.0%

Significant components of the Company s deferred tax assets at December 31 are shown below. A valuation allowance has been established as realization of such deferred tax assets has not met the more likely-than-not threshold requirement. If the Company s judgment changes and it is determined that the Company will be able to realize these deferred tax assets, the tax benefits relating to any reversal of the valuation allowance on deferred tax assets will be accounted for as a reduction to income tax expense.

	December 31,	
	2013	2012
	(in thousands)	
Deferred tax assets:		
Net operating loss carryovers	$ 79,395	$ 69,351
Deferred revenues		8,423
Research and development credits	12,253	11,257
Other comprehensive income	130	
Other	2,541	2,313
Total deferred tax assets	94,319	91,344
Deferred tax liabilities:		
Other comprehensive income		(19)
Total deferred tax liabilities		(19)
Net deferred tax asset	94,319	91,325
Valuation allowance	(94,319)	(91,325)
Net deferred tax assets	$	$

At December 31, 2013 and 2012, the Company had federal net operating loss carryforwards of approximately $192.8 million and $167.5 million, respectively. At December 31, 2013 and 2012, the Company had state net operating loss carryforwards of $181.7 million and $159.3 million, respectively. The federal and state tax loss

carryforwards will begin to expire in 2022 and 2016, respectively, unless previously utilized. At December 31, 2013 and 2012, the Company also had federal research and development tax credit carryforwards of approximately $6.4 million and $5.0 million, respectively, which will begin expiring in 2024 unless previously utilized. At December 31, 2013 and 2012, the Company also had state tax credit carryforwards of approximately $6.1 million and $5.7 million, respectively, which carry forward indefinitely. At December 31, 2013 and 2012 the Company had Canadian net operating loss carryforwards of approximately $5.9 million and $6.3 million, respectively, which begin to expire in 2026 unless previously utilized. At December 31, 2013 and 2012, the Company also had Canadian tax credits of $4.1 million and $4.4 million, respectively, which carryforward indefinitely.

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Pursuant to Internal Revenue Code (IRC), Section 382 and 383, use of the Company s U.S. federal and state net operating loss and research and development income tax credit carryforwards may be limited in the event of a cumulative change in ownership of more than 50.0% within a three-year period. The Company completed an analysis under IRC Sections 382 and 383 through December 21, 2010 and determined that the Company s net operating losses and research and development credits may be limited due to changes in ownership through December 31, 2010. Further valuation work is necessary to confirm whether or not an ownership change actually occurred during 2004 or 2005 and, because a change may have occurred, the Company has reduced its federal net operating loss carryforwards by approximately $5.5 million, its state net operating loss by $11.4 million and the federal research and development tax credit carryforwards by $2.0 million. As the Company was in a net operating loss position for the year 2013 and 2012, the Company has not performed any additional analysis for IRC Sections 382 and 383 for the years ended December 31, 2013 and 2012. There is a risk that additional changes in ownership could have occurred since that date. If a change in ownership were to have occurred, additional net operating loss and tax credit carryforwards could be eliminated or restricted. If eliminated, the related asset would be removed from the deferred tax asset schedule with a corresponding reduction in the valuation allowance.

The following table summarizes the changes in the Company s unrecognized tax benefits during the year ended December 31, 2013 (in thousands):

Gross unrecognized tax benefits at December 31, 2011	$ 2,301
Increase in prior year position	122
Increase in current year position	83
Lapse in statute of limitations	(122)
Gross unrecognized tax benefits at December 31, 2012	2,384
Increase in prior year position	176
Increase in current year position	209
Lapse in statute of limitations	(30)
Gross unrecognized tax benefits at December 31, 2013	$ 2,739

The gross unrecognized tax benefits as of December 31, 2013 were recorded as a reduction to deferred tax assets, which caused a corresponding reduction in the Company s valuation allowance. The Company does not anticipate that the amount of unrecognized tax benefits as of December 31, 2013 will change within the next twelve months. During the years ended December 31, 2013 and 2012, the Company recognized no amounts related to interest or penalties.

The Company is subject to taxation in the United States, Canada and various state and provincial jurisdictions. The Company currently has no years under examination by any jurisdiction. The Company s tax years for 2000 and forward are subject to examination by the federal and California tax authorities due to the carryforward of unutilized net operating losses and research and development credits. The Company s tax years for 2006 and forward are subject to examination by the Canadian tax authorities due to the carryforward of unutilized net operating losses and income tax credits.

10. Sale of Kinase Profiling Services Business

On October 21, 2010, the Company sold all of the assets relating to its kinase profiling service business to DiscoveRx Corporation (DiscoveRx) pursuant to an asset purchase agreement. In consideration for the sale of such assets,

DiscoveRx paid the Company $7.3 million at the closing of the transaction, $0.4 million in January 2011 and may be required to pay the Company up to an additional $4.5 million upon the achievement of certain sales and operational milestones. Under the terms of the asset purchase agreement, the Company was obligated to purchase from DiscoveRx a minimum of $625,000 of screening services during each full calendar quarter through December 31, 2012. To the extent minimum quarterly commitments exceeded the actual amount of

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services received, the Company paid the difference, which was accounted for as a reduction in both the sales price and the overall gain recorded on the sale of the business. In August 2013, the Company was notified that DiscoveRx has achieved certain, but not all, specified sales and operational milestones, resulting in the Company earning $2.5 million of the $4.5 million of incremental consideration. The Company is no longer eligible to earn the remaining $2.0 million of incremental consideration.

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

None

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our periodic and current reports that we file with the SEC is recorded, processed, summarized and reported within the time periods specified in the SEC s rules and forms, and that such information is accumulated and communicated to our management, including our chief executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. In addition, the design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, control may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

As of December 31, 2013, we carried out an evaluation, under the supervision and with the participation of our management, including our chief executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Based on this evaluation, our management, including our chief executive officer and principal financial officer, concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2013.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting identified in management s evaluation pursuant to Rules 13a-15(d) or 15d-15(d) of the Exchange Act during the quarter ended December 31, 2013 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

Not applicable.

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PART III

Certain information required by Part III of this Form 10-K is omitted from this report because registrant will file a definitive Proxy Statement within 120 days after the end of its fiscal year pursuant to Regulation 14A for its 2014 Annual Meeting of Shareholders to be held on May 15, 2014, referred to as the Proxy Statement, and the information included therein is incorporated herein by reference.

Item 10. Directors, Executive Officers and Corporate Governance

We have adopted a Code of Business Conduct and Ethics Policy that applies to our directors and employees (including our principal executive officer, principal financial officer, principal accounting officer and controller), and have posted the text of the policy on our website (www.ambitbio.com) in connection with Investor Relations materials. In addition, we intend to promptly disclose (i) the nature of any amendment to the policy that applies to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions and (ii) the nature of any waiver, including an implicit waiver, from a provision of the policy that is granted to one of these specified individuals, the name of such person who is granted the waiver and the date of the waiver on our website in the future.

The other information required by this item is incorporated by reference to the Proxy Statement under the sections entitled Election of Directors and Section 16(a) Beneficial Ownership Reporting Compliance.

Item 11. Executive Compensation

The information required by this item is incorporated herein by reference to the information from the Proxy Statement under the sections entitled Executive Compensation, Compensation Committee Report and Compensation Committee Interlocks and Insider Participation.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated herein by reference to the information from the Proxy Statement under the sections entitled Security Ownership of Certain Beneficial Owners and Management and Securities Authorized for Issuance under Equity Compensation Plans.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this item is incorporated herein by reference to the information from the Proxy Statement under the sections entitled Election of Directors and Certain Relationships and Related Transactions.

Item 14. Principal Accounting Fees and Services

The information required by this item is incorporated herein by reference to the information from the Proxy Statement under the section entitled Principal Accountant Fees and Services.

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PART IV

Item 15. Exhibits, Financial Statement Schedules

1. *Financial Statements.* The financial statements included in Item 8 are filed as part of this Annual Report on Form 10-K.

2. *Financial Statement Schedules.* All scheduled have been omitted because they are not applicable or required, or the information required to be set forth therein is included in the Financial Statements or notes thereto included in Item 8 of this Annual Report on Form 10-K.

3. *Exhibits.*

Exhibit Number	Description of Document
3.1(1)	Amended and Restated Certificate of Incorporation.
3.2(1)	Amended and Restated Bylaws.
4.1(2)	Form of Common Stock Certificate.
4.2(2)	Form of Warrant to Purchase Common Stock issued by the Company to June, July and December 2009 bridge financing investors.
4.3(2)	Form of Warrant to Purchase Common Stock issued by the Company to September 2010 bridge financing investors.
4.4(2)	Warrant issued by the Company on October 5, 2005 to Oxford Finance Corporation.
4.5(2)	Warrant issued by the Company on December 22, 2005 to Oxford Finance Corporation.
4.6(2)	Form of Warrant issued by the Company to Oxford Finance Corporation pursuant to 2006 Master Security Agreement.
4.7(2)	Form of Warrant issued by the Company to Webster Bank, National Association pursuant to 2006 Master Security Agreement.
4.8(2)	Warrant issued by the Company on October 6, 2005 to Horizon Technology Funding Company II, LLC.
4.9(2)	Warrant issued by the Company on October 6, 2005 to Horizon Technology Funding Company III, LLC.
4.10(2)	Warrant issued by the Company on September 24, 2007 to Horizon Technology Funding Company V, LLC.
4.11(2)	Warrant issued by the Company on March 31, 2010 to Compass Horizon Funding Company LLC.
4.12(2)	Warrant issued by the Company on March 31, 2010 to Oxford Finance Corporation.
4.13(2)	Form of Warrant to Purchase Series D-2 Preferred Stock issued by the Company to May 2011 Series D-2 preferred stock financing investors.

4.14(2)	Termination and Warrant Amendment Agreement dated May 18, 2012 among the Company and certain holders of Series D-2 preferred stock warrants.
4.15(2)	Second Warrant Amendment Agreement dated October 25, 2012 among the Company and certain holders of Series D-2 Preferred stock warrants.
4.16(2)	Form of Warrant to Purchase Common Stock issued by the Company to October 2012 Series E preferred stock financing investors.
4.17(2)	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Company and certain of its stockholders, as amended.

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Exhibit Number	Description of Document
4.18(3)	Termination and Amendment Agreement dated May 15, 2013 among the Company and certain of its stockholders.
10.1+	Restated Employment Agreement dated January 8, 2014 between the Company and Michael A. Martino
10.2+	Restated Employment Agreement dated January 8, 2014 between the Company and Alan Fuhrman.
10.3+	Restated Employment Agreement dated January 8, 2014 between the Company and Athena Countouriotis, M.D.
10.4(2)+	Form of Indemnity Agreement.
10.5(2)+	2011 Amended and Restated Equity Incentive Plan and forms of Stock Option Agreement thereunder, as amended.
10.6(2)+	2013 Equity Incentive Plan and form of Stock Option Agreement thereunder.
10.7(2)+	2013 Employee Stock Purchase Plan.
10.8+	Non-employee Director Compensation Policy.
10.9(2)	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Company and certain of its stockholders, as amended.
10.10(2)	Lease Agreement dated July 22, 2004 between the Company and BMR-SORRENTO VALLEY LLC, as amended.
10.11(2)	Lease Agreement dated July 17, 2012 between the Company and BMR-SORRENTO WEST LP.
10.12(2)*	Collaboration Agreement dated November 3, 2006 between the Company and Cephalon, Inc.
10.13(2)*	Collaboration Agreement dated September 14, 2010 between the Company and Genoptix, Inc.
21.1(2)	Subsidiaries of the Company.
23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney. Reference is made to the signature page.
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
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(2) Incorporated by reference to the Company s Registration Statement on Form S-1, as amended (File No. 333-186760), originally filed with the SEC on February 20, 2013.
(3) Incorporated by reference to the Company s Quarterly Report on Form 10-Q for the Quarter Ended June 30, 2013 (File No. 001-35919) originally filed with the SEC on August 13, 2013.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

AMBIT BIOSCIENCES CORPORATION

By: /s/ Michael A. Martino
Michael A. Martino,

President and Chief Executive Officer

Dated: March 20, 2014

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POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Michael Martino and Alan Fuhrman, and each of them, as his or her true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution, for him or her and in his or her name, place, and stead, in any and all capacities, to sign any and all amendments to this report, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming that all said attorneys-in-fact and agents, or any of them or their or his substitute or substituted, may lawfully do or cause to be done by virtue hereof.

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

Signature	Title	Date
/s/ Michael A. Martino	President, Chief Executive Officer and Director	
Michael A. Martino	(Principal Executive Officer)	March 20, 2014
/s/ Alan Fuhrman	Chief Financial Officer	
Alan Fuhrman	(Principal Financial and Accounting Officer)	March 20, 2014
/s/ Faheem Hasnain	Chairman of the Board, Director	
Faheem Hasnain		March 20, 2014
/s/ David Bonita, M.D.	Director	
David Bonita, M.D.		March 20, 2014
/s/ Steven A. Elms	Director	
Steven A. Elms		March 20, 2014
/s/ Standish M. Fleming	Director	
Standish M. Fleming		March 20, 2014
/s/ Mark Foletta	Director	
Mark Foletta		March 20, 2014
/s/ Allan P. Marchington, Ph.D.	Director	March 20, 2014

Allan P. Marchington, Ph.D.		
/s/ David R. Parkinson, M. D.	Director	
David R. Parkinson, M. D.		March 20, 2014
/s/ Isai Peimer	Director	
Isai Peimer		March 20, 2014

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EXHIBIT INDEX

Exhibit Number	Description of Document
3.1(1)	Amended and Restated Certificate of Incorporation.
3.2(1)	Amended and Restated Bylaws.
4.1(2)	Form of Common Stock Certificate.
4.2(2)	Form of Warrant to Purchase Common Stock issued by the Company to June, July and December 2009 bridge financing investors.
4.3(2)	Form of Warrant to Purchase Common Stock issued by the Company to September 2010 bridge financing investors.
4.4(2)	Warrant issued by the Company on October 5, 2005 to Oxford Finance Corporation.
4.5(2)	Warrant issued by the Company on December 22, 2005 to Oxford Finance Corporation.
4.6(2)	Form of Warrant issued by the Company to Oxford Finance Corporation pursuant to 2006 Master Security Agreement.
4.7(2)	Form of Warrant issued by the Company to Webster Bank, National Association pursuant to 2006 Master Security Agreement.
4.8(2)	Warrant issued by the Company on October 6, 2005 to Horizon Technology Funding Company II, LLC.
4.9(2)	Warrant issued by the Company on October 6, 2005 to Horizon Technology Funding Company III, LLC.
4.10(2)	Warrant issued by the Company on September 24, 2007 to Horizon Technology Funding Company V, LLC.
4.11(2)	Warrant issued by the Company on March 31, 2010 to Compass Horizon Funding Company LLC.
4.12(2)	Warrant issued by the Company on March 31, 2010 to Oxford Finance Corporation.
4.13(2)	Form of Warrant to Purchase Series D-2 Preferred Stock issued by the Company to May 2011 Series D-2 preferred stock financing investors.
4.14(2)	Termination and Warrant Amendment Agreement dated May 18, 2012 among the Company and certain holders of Series D-2 preferred stock warrants.
4.15(2)	Second Warrant Amendment Agreement dated October 25, 2012 among the Company and certain holders of Series D-2 Preferred stock warrants.
4.16(2)	Form of Warrant to Purchase Common Stock issued by the Company to October 2012 Series E preferred stock financing investors.
4.17(2)	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Company and certain of its stockholders, as amended.
4.18(3)	Termination and Amendment Agreement dated May 15, 2013 among the Company and certain of its

	stockholders.
10.1+	Restated Employment Agreement dated January 8, 2014 between the Company and Michael A. Martino
10.2+	Restated Employment Agreement dated January 8, 2014 between the Company and Alan Fuhrman.
10.3+	Restated Employment Agreement dated January 8, 2014 between the Company and Athena Countouriotis, M.D.

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10.4(2)+	Form of Indemnity Agreement.
10.5(2)+	2011 Amended and Restated Equity Incentive Plan and forms of Stock Option Agreement thereunder, as amended.
10.6(2)+	2013 Equity Incentive Plan and form of Stock Option Agreement thereunder.
10.7(2)+	2013 Employee Stock Purchase Plan.
10.8+	Non-employee Director Compensation Policy.
10.9(2)	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Company and certain of its stockholders, as amended.
10.10(2)	Lease Agreement dated July 22, 2004 between the Company and BMR-SORRENTO VALLEY LLC, as amended.
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