AMBIT BIOSCIENCES CORP
Form S-1/A
May 02, 2013
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As filed with the Securities and Exchange Commission on May 2, 2013

Registration No.333-186760

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Amendment No. 3

to

FORM S-1

REGISTRATION STATEMENT

UNDER

THE SECURITIES ACT OF 1933

Ambit Biosciences Corporation

(Exact name of registrant as specified in its charter)

Delaware	2834	33-0909648
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

11080 Roselle St.

San Diego, California 92121

(858) 334-2100

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

Michael A. Martino

President and Chief Executive Officer

Ambit Biosciences Corporation

11080 Roselle St.

San Diego, California 92121

(858) 334-2100

(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

Thomas A. Coll, Esq.	**Cheston J. Larson, Esq.**
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(858) 550-6000	**San Diego, CA 92130**
	(858) 523-5400

Approximate date of commencement of proposed sale to the public:

As soon as practicable after the effective date of this registration statement.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933 check the following box. ¨

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ¨

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ¨

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration number of the earlier effective registration statement for the same offering. ¨

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

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

CALCULATION OF REGISTRATION FEE

Title of each class of securities to be registered	**Proposed maximum aggregate offering price[1]**	**Amount of registration fee**
Common Stock, $0.001 par value per share	$80,126,250	$10,929[2]

(1) Estimated solely for the purpose of calculating the amount of the registration fee in accordance with Rule 457(o) under the Securities Act of 1933, as amended. Includes the offering price of shares that the underwriters have the option to purchase to cover over-allotments, if any.
(2) Previously paid.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

SUBJECT TO COMPLETION, DATED MAY 2, 2013

PRELIMINARY PROSPECTUS

4,645,000 Shares

Ambit Biosciences Corporation

Common Stock

$ per share

This is the initial public offering of our common stock. We currently expect the initial public offering price to be between $13.00 and $15.00 per share of common stock.

We have granted the underwriters an option to purchase up to 696,750 additional shares of common stock to cover over-allotments.

We have applied to list our common stock on the Nasdaq Global Market under the symbol AMBI .

Investing in our common stock involves risks. See Risk Factors beginning on page 11.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012, and, as such, we have elected to take advantage of certain reduced public company reporting requirements for this prospectus and future filings.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

	Per Share	Total
Public Offering Price	$	$
Underwriting Discount(1)	$	$
Proceeds to Ambit (before expenses)	$	$

(1) We refer you to Underwriting beginning on page 162 for additional information regarding underwriting compensation.

Certain of our existing stockholders have agreed to purchase an aggregate of $25.1 million of our common stock in a separate private placement concurrent with the completion of this offering at a price per share equal to the initial public offering price. The sale of such shares will not be registered under the Securities Act of 1933, as amended.

The underwriters expect to deliver the shares on or about , 2013 through the book-entry facilities of The Depository Trust Company.

Citigroup **Leerink Swann**

BMO Capital Markets

Baird

, 2013

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We are responsible for the information contained in this prospectus. We have not authorized anyone to provide you with different information, and we take no responsibility for any other information others may give you. If anyone provides you with different or inconsistent information, you should not rely on it. We are not, and the underwriters are not, making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should not assume that the information contained in this prospectus is accurate as of any date other than the date on the front of this prospectus.

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SUMMARY

This summary highlights information contained in other parts of this prospectus. Because it is only a summary, it does not contain all of the information that you should consider before investing in shares of our common stock and it is qualified in its entirety by, and should be read in conjunction with, the more detailed information appearing elsewhere in this prospectus. You should read the entire prospectus carefully, especially Risk Factors, Business and our audited consolidated financial statements and the related notes included at the end of this prospectus, before deciding to buy shares of our common stock. Unless the context requires otherwise, references in this prospectus to Ambit, we, us, our and the company refer to Ambit Biosciences Corporation and its subsidiaries, Ambit Biosciences (Canada) Corporation, or Ambit Canada, and Ambit Europe Limited, taken as a whole .

AMBIT BIOSCIENCES CORPORATION

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 aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, which we formerly referred to as AC220, is a once-daily, orally-administered, potent and selective inhibitor of FMS-like tyrosine kinase 3, or FLT3. Quizartinib is currently in Phase 2b clinical development in patients with relapsed/refractory acute myeloid leukemia, or AML, who express a genetic mutation in FLT3. To support a new drug application, or NDA, pending input from regulatory authorities, we plan to initiate a randomized, comparative Phase 3 clinical trial in relapsed/refractory AML patients who express a genetic mutation in FLT3 in early 2014. 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.

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, therefore, proven to be a rich source of targets for drug development with 19 approved drugs in oncology and inflammatory disease since 2001. However, the key technical limitation in the development of drugs that target kinases is the ability to design a drug that selectively inhibits the specific kinase underlying disease while minimizing activity against other kinases, or off-target activity, which can cause undesirable side effects and lead to suboptimal efficacy. 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 chemical library of approximately 8,000 compounds designed to inhibit kinases and expect to continue to leverage this library to develop viable drug candidates in the future.

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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.
- Maximize the therapeutic potential of quizartinib in AML and other hematological disease indications.
- Maximize strategic value by establishing a commercial capability to market, sell and distribute quizartinib in North America.
- Pursue strategic partnerships to accelerate development and expand the commercial opportunity for quizartinib.
- 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 discovering and developing a broad pipeline of novel drug candidates that inhibit validated kinase targets to address diseases with unmet medical need.

Our Pipeline of Targeted Therapies

We have developed a pipeline of small molecule targeted therapies using our expertise in kinase drug discovery and development. The following table summarizes this pipeline:

Quizartinib Our lead drug candidate, quizartinib, is a once-daily, orally-administered, potent and selective inhibitor of FLT3, a validated target in the treatment of AML, and is currently in Phase 2b clinical development. 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. Over 35% of AML patients over age 55 are estimated to harbor this mutation. We refer to these patients as FLT3-ITD positive. 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.

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Data from our single-arm, 333 patient Phase 2 clinical trial in relapsed/refractory AML patients was reported at the American Society of Hematology meeting in December 2012. When compared to reported results of clinical trials with other kinase inhibitors with FLT3 activity, quizartinib demonstrated superior single-agent activity in relapsed/refractory AML patients.

Our Phase 2 clinical trial demonstrated the following three key clinical benefits:

1. 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 were bridged to a potentially curative hematopoietic stem cell transplantation, or an HSCT (commonly referred to as a bone marrow transplant); and

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

In addition, nearly one of every five patients treated with quizartinib (irrespective of FLT3-ITD status) remained alive for more than 12 months and such patients are referred to as long term survivors. As of September 2012, approximately half of the long term survivors remained alive and continued to be followed for overall survival.

Since 2009, we have been developing quizartinib with a partner, Astellas Pharma Inc., and Astellas US LLC, collectively Astellas. Our agreement with Astellas will terminate effective in September 2013, following which we will own exclusive worldwide rights to quizartinib and any follow-on compounds and will be responsible for all development and commercialization activities and related costs. We and Astellas are currently in the process of developing a plan to transition the development activities currently being conducted by Astellas to us and do not anticipate that such transition will delay the clinical development activities described in this prospectus.

We are developing a companion diagnostic test with Genoptix Medical Laboratory, a Novartis company, to identify FLT3-ITD positive patients, and we believe approval of this test will be necessary for the approval of quizartinib. We plan to develop quizartinib in other AML therapeutic settings, irrespective of FLT3-ITD status, including use in newly diagnosed AML patients in combination with chemotherapy, or frontline therapy, followed by continuous single-agent maintenance therapy, as well as maintenance following an HSCT.

AC410 Our second most advanced drug candidate, AC410, is a potent, selective, orally-administered, small molecule inhibitor of JAK2, which has potential utility for the treatment of autoimmune and inflammatory diseases. Signaling through JAK controls the activation, proliferation and survival of various types of immune cells, and overactivation of such cells can exacerbate a variety of normal inflammatory processes, resulting in inflammation. 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 have selected AC410 over AC430 and AC409 for further clinical development due to its superior pharmacokinetics as observed in this clinical trial. To our knowledge, AC430 was the first selective JAK2 inhibitor to be advanced into clinical development for inflammatory disease and we believe AC410 may offer distinct benefits in this commercially attractive drug category. We plan to advance AC410 to proof-of-concept clinical trials in one or more autoimmune and inflammatory diseases, independently or in collaboration with a strategic partner.

CSF1R Program We are developing a potent and exquisitely selective small molecule compound, AC708, that inhibits CSF1R and has potential utility in oncology, autoimmune and inflammatory diseases. Signaling through CSF1R controls the activation, proliferation and survival of macrophages, which are key mediators of immune system function, and over-activation of macrophages may result in exacerbation of certain

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diseases. We have initiated investigational new drug, or IND, -enabling studies with AC708. We plan to further develop this program independently or in collaboration with a strategic partner.

Risk Factors

Our ability to implement our business strategy is subject to numerous risks and uncertainties. As a development stage biopharmaceutical company, we face many risks inherent in our business and our industry generally. You should carefully consider all of the information set forth in this prospectus and, in particular, the information under the heading Risk Factors, prior to making an investment in our common stock. These risks include, among others, the following:

- 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.

- Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and because the results of earlier studies and trials may not be predictive of future trial results, quizartinib and our other drug candidates may not achieve favorable results in ongoing or subsequent clinical trials.

- The 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.

- Our collaboration agreement with Astellas for quizartinib terminates in September 2013. In connection with the termination of this collaboration, we and Astellas must agree to a plan for transitioning all development activities from Astellas to us. If we are unable to agree to a transition plan with Astellas or if we are unable to implement any transition plan agreed upon between us and Astellas, the further development and potential commercialization of quizartinib may be delayed.

- We currently rely on a third party 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 in order to market quizartinib in the United States.

- We rely on third parties to conduct our clinical trials and to manufacture and supply quizartinib and, with respect to us, our other drug candidates, and we cannot be certain that they will successfully carry out their contractual duties or meet required timelines.

- We face significant competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively or if we fail to attain significant market acceptance for our drug candidates, if approved.

- We have a limited operating history, have incurred significant operating losses since our inception, including an accumulated deficit of $245.2 million as of March 31, 2013, and anticipate that we will continue to incur losses for the foreseeable future.

- We have no approved products and no product revenue to date, and we may never become profitable.

- 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.

If we fail to obtain additional financing, we may be unable to complete the development and commercialization of quizartinib or other drug candidates, or continue our other research and development programs.

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Concurrent Private Placement

Certain of our existing stockholders have agreed to purchase an aggregate of $25.1 million of our common stock in a separate private placement concurrent with the completion of this offering at a price per share equal to the initial public offering price. The sale of such shares will not be registered under the Securities Act of 1933, as amended.

Our Corporate Information

We were incorporated as Aventa Biosciences Corporation in Delaware in May 2000. We changed our name to Ambit Biosciences Corporation in November 2001. Our principal executive offices are located at 11080 Roselle St., San Diego, California 92121, and our telephone number is (858) 334-2100. Our website address is www.ambitbio.com. The information contained on, or that can be accessed through, our website is not a part of this prospectus. Investors should not rely on any such information in deciding whether to purchase our common stock. We have included our website address in this prospectus solely as an inactive textual reference.

We use AMBIT as a registered trademark in the United States, European Union and Japan. This prospectus also includes references to trademarks and service marks of other entities, and those trademarks and service marks are the property of their respective owners.

Emerging Growth Company

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completions of this offering, (b) in which we have total annual gross revenue of at least $1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeded $700.0 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. We refer to the Jumpstart Our Business Startups Act of 2012 herein as the JOBS Act and references herein to emerging growth company shall have the meaning associated with it in the JOBS Act.

As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- only two years of audited consolidated financial statements in addition to any required unaudited interim financial statements with correspondingly reduced Management s Discussion and Analysis of Financial Conditions and Results of Operations disclosure;
- reduced disclosure about our executive compensation arrangements;
- no requirement that we hold non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

We have taken advantage of some of these reduced burdens, and thus the information we provide stockholders may be different from what you might receive from other public companies in which you hold shares.

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THE OFFERING

Common stock offered by us	4,645,000 shares
Common stock to be sold by us to certain of our existing stockholders in the concurrent private placement, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus)	1,791,133 shares
Common stock to be outstanding after this offering and the concurrent private placement	12,889,196 shares
Over-allotment option	We have granted the underwriters an option for a period of 30 days to purchase up to 696,750 additional shares of our common stock at the initial public offering price.
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $57.6 million, or approximately $66.7 million if the underwriters exercise their over-allotment option in full, assuming an initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We also expect to receive $25.1 million from the sale by us of shares of our common stock in the concurrent private placement to certain of our existing stockholders at a price per share equal to the initial public offering price. We intend to use the net proceeds from this offering and the concurrent private placement to fund the continued clinical development of quizartinib, our lead drug candidate, to fund the continued development of our other programs and for working capital and other general corporate purposes. See Use of Proceeds on page 46 for a more complete description of the intended use of proceeds from this offering.
Risk factors	You should read the Risk Factors section of this prospectus beginning on page 11 for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Proposed Nasdaq Global Market symbol	AMBI

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The number of shares of our common stock to be outstanding after this offering and the concurrent private placement is based on 6,453,063 shares of common stock outstanding as of March 31, 2013, and excludes:

- 1,214,212 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2013, at a weighted-average exercise price of $8.75 per share;
- 6,117 shares of common stock reserved for future issuance under our 2011 amended and restated equity incentive plan (referred to herein as the 2011 pre-IPO plan) as of March 31, 2013 and an aggregate of 625,000 additional shares of common stock that will be available under our new 2013 equity incentive plan (referred to herein as our 2013 post-IPO plan), which will become effective upon the closing of this offering;
- 125,000 shares of common stock reserved for issuance under our 2013 employee stock purchase plan, or ESPP, which will become effective upon the closing of this offering; and
- 1,800,920 shares of common stock issuable upon the exercise of warrants outstanding as of March 31, 2013, at a weighted-average exercise price of $3.30 per share.

Unless otherwise noted, all information contained in this prospectus, and the number of shares of common stock outstanding as of March 31, 2013:

- reflects a 1-for-24 reverse stock split of our common stock effected on April 24, 2013;
- assumes the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior of the closing of this offering;
- assumes no exercise by the underwriters of their option to purchase up to an additional 696,750 shares of common stock to cover over-allotments;
- reflects the issuance by us of 1,538,461 shares of our Series C-2 redeemable convertible preferred stock, 612,649 shares of our Series D redeemable convertible preferred stock, 3,666,169 shares of our Series D-2 redeemable convertible preferred stock and 6,163,916 shares of our Series E redeemable convertible preferred stock prior to the closing of this offering pursuant to the exercise of the GrowthWorks put right;
- reflects the conversion of all of our outstanding shares of convertible preferred stock, including the shares issued upon exercise of the GrowthWorks put right into an aggregate of 6,449,073 shares of common stock upon the closing of this offering; and
- reflects the adjustment of outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase 645,598 shares of common stock upon the closing of this offering.

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SUMMARY CONSOLIDATED FINANCIAL INFORMATION

The following summary consolidated financial information 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 prospectus. The summary 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 and results of interim periods are not necessarily indicative of the results for the entire year.

The summary consolidated statement of operations data for the years ended December 31, 2011 and 2012 and the summary consolidated balance sheet data as of December 31, 2012 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The summary consolidated statement of operations data for the three months ended March 31, 2012 and 2013 and consolidated balance sheet data as of March 31, 2013 are derived from our unaudited consolidated financial statements and related notes appearing elsewhere in this prospectus. The unaudited consolidated financial statements have been prepared on a basis consistent with our audited consolidated financial statements included in this prospectus and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, necessary to fairly state our financial position as of March 31, 2013 and results of operations for the three months ended March 31, 2012 and 2013.

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
	(in thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Revenues:				
Collaboration agreements	$ 23,843	$ 17,633	$ 5,233	$ 6,592
Operating expenses:				
Research and development	50,705	36,731	11,140	9,005
General and administrative	8,905	6,550	1,750	1,776
Gain on sale of kinase profiling services business	(2,108)	(2,497)	(555)	
Total operating expenses	57,502	40,784	12,335	10,781
Loss from operations	(33,659)	(23,151)	(7,102)	(4,189)
Other income (expense):				
Interest expense	(4,502)	(1,737)	(356)	(162)
Other income	1,538	29		7
Change in fair value of warrant and derivative liabilities	(795)	(2,291)	(547)	(3,957)
Total other income (expense)	(3,759)	(3,999)	(903)	(4,112)
Loss before income taxes	(37,418)	(27,150)	(8,005)	(8,301)

Provision (benefit) for income taxes				1
		(121)	1	
Consolidated net loss	(37,418)	(27,029)	(8,006)	(8,302)
Net (income) loss attributable to redeemable non-controlling interest				73
	(213)	382	98	
Net loss attributable to Ambit Biosciences Corporation	(37,631)	(26,647)	(7,908)	(8,229)
Accretion to redemption value of redeemable convertible preferred stock				(2,319)
	(2,000)	(3,161)	(440)	
Change in fair value of redeemable non-controlling interest				
	4,477	(854)	(217)	(1,499)
Net loss attributable to common stockholders				$ (12,047)
	$ (35,154)	$ (30,662)	$ (8,565)	
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (25,886.60)	$ (16,591.99)	$ (6,251.82)	$ (3,019.30)
Weighted average shares outstanding, basic and diluted[1]				
			1,370	
	1,358	1,848		3,990
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]		$ (4.92)		$ (1.11)
Pro forma weighted average shares outstanding, basic and diluted (unaudited)[1]				6,422,243
		4,932,134		

(1) Please see Note 1 to our consolidated financial statements for an explanation of the method used to calculate the historical and pro forma 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 March 31, 2013		
	Actual	Pro Forma (unaudited) (in thousands)	Pro Forma As Adjusted
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 8,405	$ 8,405	$ 92,232
Working capital (deficit)	(31,873)	(26,355)	57,822
Total assets	12,424	12,424	94,778
Warrant liabilities	14,497	8,979	8,979
Notes payable, net of debt discount	3,083	3,083	3,083
Redeemable non-controlling interest	7,482		
Redeemable convertible preferred stock	159,395		
Convertible preferred stock	13,702		
Common stock		6	13
Additional paid-in capital	35,258	221,349	304,046
Accumulated deficit	(245,200)	(245,200)	(245,200)
Total stockholders deficit	(210,028)	(23,931)	58,773

The above table sets forth our summary consolidated balance sheet data as of March 31, 2013:

on an actual basis;

on a pro forma basis to give effect to:

(1) the issuance by us of 1,538,461 shares of our Series C-2 redeemable convertible preferred stock, 612,649 shares of our Series D redeemable convertible preferred stock, 3,666,169 shares of our Series D-2 redeemable convertible preferred stock and 6,163,916 shares our Series E redeemable convertible preferred stock prior to the closing of this offering pursuant to the exercise of the GrowthWorks put right and the resultant reclassification of our redeemable non-controlling interest to additional paid-in capital, a component of stockholders deficit;

(2) the conversion of all of our outstanding shares of convertible preferred stock, including the shares to be issued pursuant to the exercise of the GrowthWorks put right, into an aggregate of 6,449,073 shares of common stock upon the closing of this offering;

(3) the adjustment of our outstanding warrants to purchase convertible preferred stock into warrants to purchase 645,598 shares of common stock upon the closing of this offering, and the resultant reclassification of our redeemable convertible preferred stock warrant liabilities to additional paid-in capital, a component of stockholders deficit; and

on a pro forma as adjusted basis to additionally give effect to the sale by us in the concurrent private placement to certain of our existing stockholders of $25.1 million of our common stock and the sale of 4,645,000 shares of common stock in this offering, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase (decrease) in the assumed initial public offering price would increase (decrease) each of the cash and cash equivalents, working capital, total assets and total stockholders deficit by $4.3 million, assuming the number of shares offered by us as stated on the cover page of this prospectus remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, a one million share increase (decrease) in the number of shares offered by us, as set forth on the cover page of this prospectus,

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would increase (decrease) each of cash and cash equivalents, working capital, total assets and total stockholders deficit by $13.0 million, assuming the assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this prospectus, including our audited consolidated financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in our common stock. If any of the following risks actually occurs, our business, prospects, financial condition and results of operations could suffer materially, the trading price of our common stock could decline and you could lose all or part of your investment.

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 quizartinib, our lead drug candidate, for which a Phase 2 clinical trial and a Phase 2b clinical trial are ongoing. 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 expect, pending regulatory authority input, to initiate a randomized, comparative Phase 3 clinical trial of quizartinib in patients with relapsed/refractory acute myeloid leukemia, or AML, in early 2014. There is no guarantee that this trial will commence or be completed on time or at all. Even if the trial is successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do. To the extent that 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 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.

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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 completed enrollment for a Phase 2 clinical trial of quizartinib for the treatment of AML in late 2011. We have been collaborating with Astellas Pharma Inc. and Astellas US LLC, or collectively Astellas, on the development of quizartinib. Astellas is currently conducting a Phase 2b clinical trial to determine the optimal dose for a Phase 3 clinical trial that we are planning to initiate in early 2014 in patients with relapsed/refractory AML. We have not yet finalized the design of this trial or received feedback on the proposed study design from the FDA. The Phase 3 clinical trial design will be based on data from the Phase 2 trial, the ongoing Phase 2b clinical trial and an ongoing drug-drug interaction study and on guidance we will seek from the FDA. The FDA may require us to conduct additional studies before proceeding with the Phase 3 clinical trial.

Our collaboration with Astellas for the development of quizartinib terminates in September 2013. Any interruptions or delays in transitioning full responsibility for clinical development to us in connection with such termination could delay the commencement of the Phase 3 clinical trial. In addition, any delays in obtaining data from the drug-drug interaction study or in the completion of the Phase 2b clinical trial could delay the commencement of the Phase 3 clinical trial. We are currently evaluating quizartinib in two 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 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;
- 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 Astellas, 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.

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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 our drug candidates, the commercial prospects of our drug candidates will be harmed, and our ability to generate product revenues from any of these drug candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our 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;
- 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;

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- 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; 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.

Our collaboration agreement with Astellas for quizartinib terminates in September 2013. In connection with the termination of this collaboration, we and Astellas must agree to a plan for transitioning all development activities from Astellas to us. If we are unable to agree to a transition plan with Astellas or if we are unable to implement any transition plan agreed upon between us and Astellas, the further development and potential commercialization of quizartinib may be delayed.

In March 2013, Astellas exercised the right to terminate our collaboration agreement, effective in September 2013, under which we collaborated with Astellas for the development of quizartinib and under which we and Astellas shared agreed-upon development costs equally. As a result of the termination of our collaboration, we will be solely responsible for developing and commercializing quizartinib within the United States and the rest of the world and will be responsible for many of the functions previously expected to be Astellas responsibility, including management and oversight of certain ongoing clinical trials and the planned Phase 3 clinical trial, as well as submitting the NDA for quizartinib to the FDA. We and Astellas are in the process of developing a plan to transition the development activities currently being conducted by Astellas to us. Currently, no such plans have been agreed upon and we cannot assure you that our efforts to transition Astellas collaboration responsibilities will proceed on a timely basis, or at all. If we are unable to successfully transition Astellas quizartinib development activities to us on a timely basis, our development plans may be delayed, which could harm our business, prospects, financial condition and results of operations.

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 AML patients with internal tandem duplication, or ITD, mutations in the FMS-like tyrosine kinase 3, or FLT3, gene, which we refer to as FLT3-ITD positive. 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, it is expected that the Phase 3 trial designed to support marketing approval for quizartinib will use 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

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product. We believe a companion diagnostic to test for the FLT3-ITD positive genotype will be required for the approval of quizartinib. 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 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 400 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

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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 prolongation for distribution to patients and a communication plan to health care practitioners. 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

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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 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.

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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-marketing testing, including 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;
- 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 Phase 2 clinical trials for quizartinib. We anticipate that we will engage one or more third party 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

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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 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.

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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 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 equivalent 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.

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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 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

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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 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.

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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 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,

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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 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.

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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;
- 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

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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.

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 March 31, 2013, we employed 45 employees, 41 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, 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.

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;

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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.

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

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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;
- 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.

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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 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.

To date, we have financed our consolidated operations primarily through private placements of convertible debt and preferred stock, venture debt and our collaboration and license arrangements, and we have incurred significant operating losses since our inception, including consolidated net losses of $37.4 million and $27.0 million for the years ended December 31, 2011 and 2012, respectively, and $8.3 million for the three months ended March 31, 2013. As of March 31, 2013, we had an accumulated deficit of $245.2 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.

Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in this prospectus.

Our report from our independent registered public accounting firm for the year ended December 31, 2012 includes an explanatory paragraph stating that our recurring losses from operations and negative cash flows raise substantial doubt about our ability to continue as a going concern. If we are unable to obtain sufficient funding, our business, prospects, financial condition and results of operations will be materially and adversely affected and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited consolidated financial statements, and it is likely that investors will lose all or a part of their investment. After this offering, future reports from our independent registered public accounting firm may also contain statements expressing doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding on commercially reasonable terms or at all.

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

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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.

The terms of our secured debt facility require us to meet certain operating and financial covenants and place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.

On March 31, 2010, we entered into a $12.0 million venture loan and security agreement, or the Compass Loan, with Compass Horizon Funding Company LLC and Oxford Finance Corporation, collectively, the Lenders. The Compass Loan is secured by a lien covering substantially all of our assets, excluding intellectual property, and we also pledged as collateral a portion of our equity interests in our subsidiaries. The Compass Loan is amortizing and we are obligated to make monthly payments of principal and interest through the maturity date of October 1, 2013, assuming there is no default that results in acceleration of the debt. As of March 31, 2013, the outstanding principal balance of the Compass Loan was $3.1 million.

The loan agreement governing the Compass Loan contains customary affirmative and negative covenants and events of default. The affirmative covenants include, among others, covenants requiring us to maintain our legal existence and governmental approvals, deliver certain financial reports and maintain insurance coverage. The negative covenants include, among others, restrictions on transferring collateral, changing our business, incurring additional indebtedness, engaging in mergers or acquisitions, paying dividends or making other distributions, making investments and creating other liens on our assets, in each case subject to customary exceptions. If we default under the Compass Loan, the Lenders may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Further, if we are liquidated, the Lenders right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. The Lenders could declare a default under the Compass Loan upon the occurrence of any event that they interpret as a material adverse change as defined under the loan agreement, thereby requiring us to repay the loan immediately or to attempt to reverse the declaration of default through negotiation or litigation. Any declaration by the Lenders of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

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If we fail to obtain additional financing, we may be unable to complete the development and commercialization of quizartinib or other drug candidates, or continue our other research and development programs.

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 ongoing Phase 2 and Phase 2b clinical trials and planned Phase 3 clinical trial, as well as the preclinical and clinical development of other drug candidates;
- continue our research and development programs to advance our internal product pipeline;
- 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; and
- service and/or repay the Compass Loan.

We estimate that our net proceeds from this offering and the concurrent private placement will be approximately $82.7 million, based upon an assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and offering expenses payable by us. We believe that such proceeds together with our existing cash and cash equivalents, will be sufficient to fund our operations through at least the next 12 months. In particular, we believe that the net proceeds from this offering and the concurrent private placement intended for clinical development of quizartinib and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund such development through receipt of topline data from our planned Phase 3 clinical trial in patients with relapsed/refractory AML. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. 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.

We cannot be certain that additional funding will be available on acceptable terms, or at all. Subject to limited exceptions, the Compass Loan prohibits us from incurring additional indebtedness. If we are unable to raise additional capital 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 one or more of our 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 technologies or 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.

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

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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 this offering, the concurrent private placement, our most recent private placement and other transactions that have occurred over the past three years, we have experienced, or may upon completion of this offering experience, 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, 2012, we had federal and state net operating loss carryforwards of approximately $151.2 million and $143.1 million, respectively, and federal research and development credits of $5.0 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 March 31, 2013, we had $8.4 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 March 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.

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.

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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 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.

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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, 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.

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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 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

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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 This Offering and Ownership of our Common Stock

We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.

Prior to this offering there has been no public market for shares of our common stock. Although we expect that our common stock will be approved for listing on the Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in our common shares is not active. The initial public offering price for our common stock will be determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of the common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

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

The trading price of our common stock following this offering is likely 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 termination of a collaboration or 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. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. 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, the Compass Loan currently prohibits us from paying dividends on our equity securities, and 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.

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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.

Prior to this offering, our executive officers, directors, 5% stockholders and their affiliates owned approximately 93.7% of our voting stock and, upon the closing of this offering and the concurrent private placement, that same group will hold approximately 60.0% of our outstanding voting stock (assuming no exercise of the underwriters over-allotment option) in each case assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). Therefore, even after this offering 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.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price is substantially higher than the net tangible book value per share of our common stock. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the book value of our tangible assets after subtracting our liabilities. As a result, investors purchasing common stock in this offering will incur immediate dilution of $9.44 per share, based on an initial public offering price of $14.00 per share. Further, investors purchasing common stock in this offering will contribute approximately 22% of the total amount invested by stockholders since our inception, but will own approximately 36% of the shares of common stock outstanding after giving effect to this offering.

In addition, as of March 31, 2013, options to purchase 1,214,212 shares of our common stock at a weighted-average exercise price of $8.75 per share and warrants exercisable for up to 1,800,920 shares of our common stock at a weighted-average price of $3.30 per share were outstanding. The exercise of any of these options or warrants would result in additional dilution. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see Dilution.

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

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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.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, which will require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. 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, 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 pricing of this offering. 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.

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

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of

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March 31, 2013, upon the closing of this offering and the concurrent private placement, we will have outstanding a total of 12,889,196 shares of common stock, assuming no exercise of the underwriters overallotment option and no exercise of outstanding options and warrants. Of these shares, only the 4,645,000 shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters overallotment option, will be freely tradable without restriction in the public market immediately following this offering. Citigroup Global Markets Inc. and Leerink Swann LLC, however, may, in their sole discretion, permit our officers, directors and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

We expect that the lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus. After the lock-up agreements expire, up to an additional 8,244,196 shares of common stock will be eligible for sale in the public market, 4,479,229 of which shares are held by directors, executive officers and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended, or the Securities Act, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). In addition, 1,970,329 shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

After this offering, the holders of 5,005,963 shares of our common stock, or approximately 77.6% of our total outstanding common stock as of March 31, 2013, will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above and assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). See Description of Capital Stock Registration Rights. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

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, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock, including shares of common stock sold in this offering.

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.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have

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experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management s attention and resources, which could harm our business.

We have broad discretion in the use of the net proceeds from this offering and the concurrent private placement and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and the concurrent private placement, including for any of the purposes described in the section entitled Use of Proceeds, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our net proceeds in ways that ultimately increase the value of your investment. We expect to use the net proceeds from this offering and the concurrent private placement to fund the continued development of quizartinib, to fund the continued development of our other programs and for working capital and other general corporate purposes. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering and the concurrent private placement in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

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

Provisions in our 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;
- 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.

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If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. The forward-looking statements are contained principally in the sections entitled Summary, Risk Factors, Management s Discussion and Analysis of Financial Condition and Results of Operations, and Business . These statements relate to future events or to our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. The words may, will, could, would, should, expect, intend, plan, anticipate, belie predict, project, potential, continue, ongoing or the negative of these terms or other similar expressions are intended to identify forward-look statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus include, among other things, statements about:

- the success and timing of our preclinical studies and clinical trials, including our ongoing Phase 2 and Phase 2b clinical trials and planned Phase 3 clinical trial for quizartinib;
- our ability to obtain and maintain regulatory approval of our drug candidates, including quizartinib, and the labeling under any approval we may obtain;
- our plans to develop and commercialize quizartinib and our other drug candidates, including our plan to enter into a transition agreement with Astellas for development activities of quizartinib and our plan to establish our own commercial capabilities;
- the performance of our existing collaborators, including Genoptix, Inc. and Teva Pharmaceutical Industries Ltd., and our ability to maintain such collaborations;
- our ability to leverage our proprietary chemical library to develop viable drug candidates;
- our ability to establish additional collaborations for our drug candidates;
- the loss of key scientific or management personnel;
- the size and growth of the potential markets for quizartinib and our other drug candidates and our ability to serve those markets;
- regulatory developments in the United States and foreign countries;
- the rate and degree of market acceptance of any approved products;
- our expectations regarding the period during which we will be an emerging growth company under the JOBS Act;

our use of the proceeds from this offering, and the clinical milestones we expect to fund with such proceeds;

the accuracy of our estimates regarding expenses, future revenues and capital requirements;

our ability to obtain funding for our operations;

our ability to obtain and maintain intellectual property protection for our drug candidates and our ability to operate our business without infringing on the intellectual property rights of others;

the success of competing drugs that are or become available; and

the performance of third-party manufacturers and clinical research organizations.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. You should refer to the Risk Factors section of this prospectus for a discussion of important factors

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that may cause actual results or events to differ materially from those expressed or implied by our forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments we may make. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results or events to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. The Private Securities Litigation Reform Act of 1995 and Section 27A of the Securities Act, do not protect any forward-looking statements that we make in connection with this offering.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

This prospectus also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

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USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of 4,645,000 shares of common stock in this offering will be approximately $57.6 million. We expect to also receive $25.1 million from the sale by us of shares of our common stock in the concurrent private placement to certain of our existing stockholders at a price per share equal to the initial public offering price, for an aggregate amount to be raised by us in this offering and the concurrent private placement of $82.7 million, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share would increase (decrease) our expected net proceeds from this offering by approximately $4.3 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their over-allotment option in full, we estimate that the net proceeds from this offering and the concurrent private placement will be approximately $91.8 million, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The principal purposes of this offering are to obtain additional capital to support our operations, to create a public market for our common stock and to facilitate our future access to the public equity markets. We intend to use the net proceeds from this offering and the concurrent private placement as follows:

- approximately $61.0 million to fund the continued clinical development of quizartinib;
- approximately $3.0 million to fund the continued development of our other programs; and
- the remainder for working capital and other general corporate purposes.

We believe that the net proceeds from this offering and the concurrent private placement and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations through at least the next 12 months. In particular, we believe that the net proceeds from this offering and the concurrent private placement intended for clinical development of quizartinib and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund such development through receipt of topline data from our planned Phase 3 clinical trial in patients with relapsed/refractory AML.

The expected use of the net proceeds from this offering and the concurrent private placement represents our intentions based upon our current plans and business conditions. 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 this offering 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. Pending use of the proceeds from this offering and the concurrent private placement, we intend to invest the proceeds in a variety of short-term, investment-grade and interest-bearing instruments.

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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 on, 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. In addition, unless waived, the terms of our Venture Loan and Security Agreement with Compass Horizon Funding Company LLC and Oxford Finance Corporation prohibit us from paying dividends on our common stock.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of March 31, 2013:

- on an actual basis;
- on an a pro forma basis to give effect to:
  - the issuance by us of 1,538,461 shares of our Series C-2 redeemable convertible preferred stock, 612,649 shares of Series D redeemable convertible preferred stock, 3,666,169 shares of our Series D-2 redeemable convertible preferred stock and 6,163,916 shares our Series E redeemable convertible preferred stock prior to the closing of this offering pursuant to the exercise of a put right held by GrowthWorks Canadian Fund Ltd., or the GrowthWorks put right, and the resultant reclassification of our redeemable non-controlling interest to additional paid-in capital, a component of stockholders deficit;
  - the conversion of all of our outstanding shares of convertible preferred stock, including the shares to be issued pursuant to the exercise of the GrowthWorks put right, into an aggregate of 6,449,073 shares of common stock upon the closing of this offering;
  - the adjustment of outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase 645,598 shares of common stock upon the closing of this offering, and the resultant reclassification of our redeemable convertible preferred stock warrant liabilities to additional paid-in capital, a component of stockholders deficit; and
  - the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws as of the closing date of this offering.
- on a pro forma as adjusted basis to additionally give effect to the sale by us in the concurrent private placement to certain of our existing stockholders of $25.1 million of our common stock and the sale of 4,645,000 shares of common stock in this offering, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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The pro forma as adjusted information below is illustrative only and our cash and cash equivalents and capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this table together with our audited consolidated financial statements and the related notes appearing at the end of this prospectus, the sections entitled Selected Consolidated Financial Data and Management s Discussion and Analysis of Financial Condition and Results of Operations and other financial information contained in this prospectus.

	As of March 31, 2013		
	Actual	Pro Forma (unaudited)	Pro Forma As Adjusted[1]
	(in thousands except share and per share data)		
Cash and cash equivalents	$ 8,405	$ 8,405	$ 92,232
Capitalization:			
Warrant liabilities	$ 14,497	$ 8,979	$ 8,979
Redeemable non-controlling interest	7,482		
Notes payable, net of debt discount	3,083	3,083	3,083
Preferred stock, $0.001 par value: 170,990,763 shares authorized; 123,416,438 shares issued and outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted	173,097		
Common stock, $0.001 par value: 225,000,000 shares authorized; 3,990 shares issued and outstanding, actual; 200,000,000 shares authorized and 6,453,063 shares issued and outstanding, pro forma; 200,000,000 shares authorized and 12,889,196 shares issued and outstanding, pro forma as adjusted		6	13
Additional paid-in capital	35,258	221,349	304,046
Accumulated other comprehensive income	(86)	(86)	(86)
Accumulated deficit	(245,200)	(245,200)	(245,200)
Total stockholders deficit	(210,028)	(23,931)	58,773
Total capital deficit	$ (11,869)	$ (11,869)	$ 70,835

(1) A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share (which is the midpoint of the price range set forth on the cover page of this prospectus) would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders deficit and total capitalization by approximately $4.3 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The number of shares in the table above excludes, as of March 31, 2013:

1,214,212 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2013, at a weighted-average exercise price of $8.75 per share;

6,117 shares of common stock reserved for future issuance under our 2011 pre-IPO plan as of March 31, 2013 and an aggregate of 625,000 additional shares of common stock that will be available under our 2013 post-IPO plan, which will become effective upon the closing of this offering;

125,000 shares of common stock reserved for issuance under our 2013 employee stock purchase plan, or ESPP, which will become effective upon the closing of this offering; and

1,800,920 shares of common stock issuable upon the exercise of warrants outstanding as of March 31, 2013, at a weighted-average exercise price of $3.30 per share.

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DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book deficit of our common stock as of March 31, 2013 was approximately $(210.0) million, or $(52,638.60) per share, based on the 3,990 shares of common stock outstanding as of March 31, 2013. Historical net tangible book deficit per share is determined by dividing the number of shares of common stock outstanding as of March 31, 2013 into our total tangible assets (total assets less intangible assets) less total liabilities and convertible preferred stock.

On a pro forma basis, after giving effect to the conversion of all outstanding shares of convertible preferred stock into 6,449,073 shares of common stock, including the shares of redeemable convertible preferred stock to be issued pursuant to the exercise of the GrowthWorks put right, with the resulting reclassification of our redeemable non-controlling interest to additional paid-in capital, a component of stockholders deficit, and the reclassification of our redeemable convertible preferred stock warrant liabilities to additional paid-in capital, our net tangible book deficit as of March 31, 2013 would have been approximately $(23.9) million, or approximately $(3.71) per share.

Investors participating in this offering will incur immediate, substantial dilution. After giving effect to (1) the sale by us of shares of common stock in the concurrent private placement to certain of our existing stockholders at an assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), (2) the sale of common stock offered by us in this offering at an assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, and (3) the pro forma transactions described in the preceding paragraph, our pro forma as adjusted net tangible book value as of March 31, 2013 would have been approximately $58.8 million, or approximately $4.56 per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of $8.27 per share to existing stockholders, and an immediate dilution of $9.44 per share to investors participating in this offering. The following table illustrates this per share dilution to investors participating in this offering:

Assumed initial public offering price per share		$ 14.00
Historical net tangible book deficit per share as of March 31, 2013	$ (52,638.60)	
Pro forma decrease in net tangible book deficit per share attributable to pro forma transactions described in preceeding paragraphs	52,634.89	
Pro forma net tangible book value (deficit) per share as of March 31, 2013	(3.71)	
Pro forma increase in net tangible book value per share attributable to investors participating in this offering	8.27	
Pro forma as adjusted net tangible book value per share after this offering		4.56
Pro forma dilution per share to investors participating in this offering		$ 9.44

A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) would increase (decrease) our pro forma as adjusted net tangible book value as of March 31, 2013 by approximately $4.3 million, our pro forma as adjusted net tangible book value per share after this offering by $0.34 and the dilution in pro forma as adjusted net tangible book value to investors participating in this offering by $0.34 per share, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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If the underwriters exercise their over-allotment option in full to purchase 696,750 additional shares of common stock in this offering, our pro forma as adjusted net tangible book value per share after the offering and concurrent private placement would be $4.99 per share, the increase in the pro forma net tangible book value per share to existing stockholders would be $8.70 per share and the dilution to investors participating in this offering would be $9.01 per share.

The following table summarizes, on the pro forma as adjusted basis described above as of March 31, 2013, the differences between the number of shares of common stock purchased from us by existing stockholders, which includes the concurrent private placement to certain of our existing stockholders, and by new investors participating in this offering, the total consideration and the average price per share paid to us by existing stockholders and by investors participating in this offering, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus):

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percentage	Amount	Percentage	
Existing stockholders before this offering	8,244,196	64%	$ 235,999,314	78%	$ 28.63
Investors participating in this offering	4,645,000	36%	65,030,000	22%	14.00
Total	12,889,196	100%	$ 301,029,314	100%	

A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) would increase (decrease) total consideration paid to us by investors participating in this offering by approximately $4.3 million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Except as otherwise indicated, the discussion and tables above assume no exercise of the underwriters over-allotment option or any outstanding options or warrants. If the underwriters over-allotment option is exercised in full, the number of shares of common stock held by existing stockholders will be reduced to 61% of the total number of shares of common stock to be outstanding after this offering, and the number of shares of common stock held by investors participating in this offering will be further increased to 5,341,750, or 39% of the total number of shares of common stock to be outstanding after this offering.

The number of shares in the table above excludes, as of March 31, 2013:

- 1,214,212 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2013, at a weighted-average exercise price of $8.75 per share;
- 6,117 shares of common stock reserved for future issuance under our 2011 pre-IPO plan as of March 31, 2013 and an aggregate of 625,000 additional shares of common stock that will be available under our 2013 post-IPO plan, which will become effective upon the closing of this offering;
- 125,000 shares of common stock reserved for issuance under our 2013 employee stock purchase plan, or ESPP, which will become effective upon the closing of this offering; and
- 1,800,920 shares of common stock issuable upon the exercise of warrants outstanding as of March 31, 2013, at a weighted-average exercise price of $3.30 per share.

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Effective immediately upon the signing of the underwriting agreement for this offering, an aggregate of 631,117 and 125,000 shares of our common stock will be reserved for future issuance under our 2013 post-IPO plan and ESPP, respectively, which includes 6,117 shares of common stock reserved for future issuance under our 2011 pre-IPO plan as of March 31, 2013 that will be allocated to our 2013 post-IPO plan, and these share reserves will also be subject to automatic annual increases in accordance with the terms of the plans. Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that any of these options or warrants is exercised, new options are issued under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future, there will be further dilution to investors participating in this offering.

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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 prospectus. 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 future results that may be expected in the future and results of interim periods are not necessarily indicative of the results for the entire year.

The selected consolidated statement of operations data for the years ended December 31, 2011 and 2012 and the selected consolidated balance sheet data as of December 31, 2011 and 2012 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The selected consolidated statement of operations data for the three months ended March 31, 2012 and 2013 and the selected consolidated balance sheet data as of March 31, 2013 are derived from our unaudited consolidated financial statements appearing elsewhere in this prospectus. The unaudited consolidated financial statements have been prepared on a basis consistent with our audited consolidated financial statements included in this prospectus and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, necessary to fairly state our financial position as of March 31, 2013 and results of operations for the three months ended March 31, 2012 and 2013.

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
	(in thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Revenues:				
Collaboration agreements	$ 23,843	$ 17,633	$ 5,233	$ 6,592
Operating expenses:				
Research and development	50,705	36,731	11,140	9,005
General and administrative	8,905	6,550	1,750	1,776
Gain on sale of kinase profiling services business	(2,108)	(2,497)	(555)	
Total operating expenses	57,502	40,784	12,335	10,781
Loss from operations	(33,659)	(23,151)	(7,102)	(4,189)
Other income (expense):				
Interest expense	(4,502)	(1,737)	(356)	(162)
Other income	1,538	29		7
Change in fair value of warrant and derivative liabilities	(795)	(2,291)	(547)	(3,957)
Total other income (expense)	(3,759)	(3,999)	(903)	(4,112)
Loss before income taxes	(37,418)	(27,150)	(8,005)	(8,301)
Provision (benefit) for income taxes		(121)	1	1
Consolidated net loss	(37,418)	(27,029)	(8,006)	(8,302)
Net (income) loss attributable to redeemable non-controlling interest	(213)	382	98	73
Net loss attributable to Ambit Biosciences Corporation	(37,631)	(26,647)	(7,908)	(8,229)
Accretion to redemption value of redeemable convertible preferred stock	(2,000)	(3,161)	(440)	(2,319)
Change in fair value of redeemable non-controlling interest	4,477	(854)	(217)	(1,499)
Net loss attributable to common stockholders	$ (35,154)	$ (30,662)	$ (8,565)	$ (12,047)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (25,886.60)	$ (16,591.99)	$ (6,251.82)	$ (3,019.30)

Weighted average shares outstanding, basic and diluted[1]			1,370
	1,358	1,848	3,990
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]		$ (4.92)	$ (1.11)
Pro forma weighted average shares outstanding, basic and diluted (unaudited)[1]		4,932,134	6,422,243

(1) Please see Note 1 to our consolidated financial statements for an explanation of the method used to calculate the historical and pro forma 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,		As of March 31,
	2011	2012	2013
		(in thousands)	
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 16,417	$ 17,481	$ 8,405
Working capital (deficit)	(6,023)	(11,113)	(31,873)
Total assets	22,820	19,989	12,424
Notes payable, net of debt discount	8,911	4,320	3,083
Warrant liabilities	4,916	10,540	14,497
Redeemable non-controlling interest	1,322	3,323	7,482
Redeemable convertible preferred stock	132,340	157,076	159,395
Convertible preferred stock	13,752	13,702	13,702
Accumulated deficit	(210,324)	(236,971)	(245,200)
Total stockholders deficit	(177,364)	(198,246)	(210,028)

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MANAGEMENT S DISCUSSION AND ANALYSIS OF

FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our audited consolidated financial statements and the related notes appearing at the end of this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the Risk Factors section of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

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 aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, which we formerly referred to as AC220, is a once-daily, orally-administered, potent and selective inhibitor of the FMS-like tyrosine kinase 3, or FLT3. Quizartinib is currently in Phase 2b clinical development in patients with relapsed/refractory acute myeloid leukemia, or AML, who express a genetic mutation in FLT3. 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 $37.4 million and $27.0 million in the years ended December 31, 2011 and 2012, respectively, and $8.3 million for the three months ended March 31, 2013. As of March 31, 2013, we had an accumulated deficit of $245.2 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 March 31, 2013, we had cash and cash equivalents of

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$8.4 million. Although it is difficult to predict future liquidity requirements, we believe that the net proceeds from this offering and the concurrent private placement and 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 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. In its report on our financial statements for the year ended December 31, 2012, our independent registered public accounting firm included an explanatory paragraph expressing doubt regarding our ability to continue as a going concern.

We conduct the majority of our activities through Ambit Biosciences Corporation, a Delaware corporation, from our primary facility in San Diego, California. Additionally, as of March 31, 2013, we owned 36% of the outstanding capital stock, and 50% of the voting stock, of Ambit Canada which in the past conducted limited research and development activities in Toronto. We also have a wholly-owned subsidiary, Ambit Europe Limited, located in the United Kingdom, which 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.

Collaboration Agreements

Astellas

In December 2009, we entered into a worldwide agreement with Astellas to jointly research, develop and commercialize certain FLT3 kinase inhibitors. In March 2013, we received a notice of termination of the agreement from Astellas, which termination will be effective in September 2013. 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 share 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. Under the agreement, Astellas was responsible for development and research costs for quizartinib in other geographic markets. Following the effective date of the termination, we will own all rights to quizartinib and any follow-on compounds and will be 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.

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 received two milestone payments totaling $3.0 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 $47.5 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

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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 reimbursement from Astellas through the effective date of the termination of our agreement and 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 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

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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):

	Years Ended December 31,		Three Months Ended March 31,		Total January 1, 2007 through March 31,
	2011	2012	2012	2013	2013
Quizartinib	$ 35,491	$ 26,880	$ 8,260	$ 6,992	$ 112,418
AC410 /AC430	3,723	792	457	38	15,661
CSF1R	2,739	1,440	469	351	11,680
Discovery projects	4,866	4,625	1,119	672	59,215
R&D administration	3,886	2,994	835	952	14,364
Total	$ 50,705	$ 36,731	$ 11,140	$ 9,005	$ 213,338

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 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 and Astellas complete the ongoing clinical trials of quizartinib and we 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.

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;

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- 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.

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. We expect these increases to be partially offset by a reduction in costs related to a facility move completed in the first quarter of 2013.

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.

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 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

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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.

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

In October 2009, the Financial Accounting Standards Board, or 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. 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.

Pursuant to the new standard, each required deliverable 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

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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 from milestones is accounted for in accordance with Accounting Standards Codification, or ASC, 605-28, *Revenue Recognition Milestone Method*. 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.

Stock-Based Compensation

We account for stock-based compensation by measuring and recognizing compensation expense for all stock-based payments made to employees and 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 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.

The following table summarizes our weighted-average assumptions used in the Black-Scholes model to value employee option grants. No data is presented in the table below for the three months ended March 31, 2013 since we granted no equity awards during that period.

	Years Ended December 31,		Three Months Ended
	2011	2012	March 31, 2012
Risk-free interest rate	1.2%	0.9%	1.3%
Expected dividend yield			
Expected volatility	63.1%	67.4%	64.8%
Expected term (in years)	6.1	6.0	6.1

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.

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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.

Common Stock Value

From inception through December 31, 2012, due to the absence of an active market for our common stock, we had determined the exercise prices for all options granted based on the estimated fair value as determined contemporaneously on the date of grant by our board of directors, with input from management. All options to purchase shares of our common stock are intended to be granted with an exercise price per share no less than the fair value per share of our common stock underlying those options on the date of grant, based on the information known to us on the date of grant.

In connection with the preparation of the consolidated financial statements necessary for inclusion in the registration statement related to this offering, we reassessed the estimated fair value of our common stock during each quarterly period in 2011, 2012 and 2013. Our reassessments of the fair value of our common stock were done using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, or AICPA, Audit and Accounting Practice Aid Series: *Valuation of Privately Held Company Equity Securities Issued as Compensation*, or the AICPA Practice Guide. In addition, our board of directors considered various objective and subjective factors to determine the fair value of our common stock, including: external market conditions affecting the biopharmaceutical industry, trends within the biopharmaceutical industry, the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant, our results of operations and financial position, status of our research and development efforts, our stage of development and business strategy, the lack of an active public market for our common and our preferred stock, and the likelihood of achieving a liquidity event such as an initial public offering, or IPO, or sale of our company in light of prevailing market conditions. The reassessments included both the determination of the appropriate valuation model and related inputs. We concluded that the reassessed fair value of our common stock was at or below the exercise price of options granted during each quarterly period in 2011 and 2012 and we did not grant any equity awards during the quarter ended March 31, 2013. As a result, no incremental fair value was added to the stock based compensation recorded for options issued from January 1, 2011 to December 31, 2012.

Our reassessment analyses were based on a methodology that first estimated the fair value of our business as a whole, or enterprise value. The determination of enterprise value was based on two primary factors: (i) a market approach using IPO comparables; and (ii) a market approach using mergers and acquisitions, or M&A, transaction comparables. Once we determined the expected enterprise value we then adjusted for expected cash and debt balances, allocated value to the various stockholders, adjusted to present value and discounted for lack of marketability.

During 2011, 2012 and 2013, we utilized the probability weighted expected return method, or PWERM, to allocate estimated enterprise value to our common stock. The PWERM considers the present value of the returns afforded to stockholders under each expected liquidity event at each valuation date. These scenarios varied over time as to timing and probability and generally consisted of: (i) IPOs; (ii) M&A or reverse merger transactions; and (iii) liquidation. The timing of the future liquidity event scenarios was determined based primarily on input from our board of directors and management. Under the IPO scenarios, value was allocated on a fully diluted basis while the sale scenarios took into account the conversion rights and liquidation preferences of each class of stock. Under each scenario the option and warrant holders were assumed to exercise to the extent the exercise prices of their options and warrants were below the estimated fair value of the underlying securities. The resulting values were then adjusted to present value based on the estimated time to liquidity, discounted for lack of marketability, and then probability weighted based on our estimate of the likelihood and timing of each liquidity scenario considered at each valuation date.

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The key subjective factors and assumptions used in our valuations primarily consisted of: (i) the selection of the appropriate valuation model, (ii) the selection of the appropriate market comparable transactions, (iii) the financial forecasts utilized to determine future cash balances and necessary capital requirements, (iv) the probability and timing of the various possible liquidity events, (v) the estimated weighted average cost of capital and (vi) the discount for lack of marketability of our common stock.

Given the limited number of biopharmaceutical IPOs and public and private M&A transactions from 2010 through March 31, 2013, we considered the population of all biopharmaceutical IPOs and all public and private biopharmaceutical M&A transactions, beginning with transactions in 2010 and including transactions through the related valuation date, across all stages of development and therapeutic areas. Comparables in Phase 3 clinical development (or later) were removed as we had an expectation that our IPO date and/or M&A transaction date would occur while our lead drug candidate was still in Phase 2 clinical development. Certain very large and very small transactions were eliminated as outliers. The remaining comparables provided a wide range of therapeutic areas, addressable markets and stages of development. During 2011 and 2012, we generally selected either the low-end, average or high-end of the comparables based on our assessment of which valuation was most likely, given the timing of each event, our expected development progress and financial condition at the expected liquidity event date. During the first quarter of 2013, we selected a higher level of comparable transactions as we progressed towards our IPO and achieved certain operational and business milestones, as further discussed below.

At each valuation date we used our then current board of directors approved budget or forecast to determine our estimated financing needs and forecasted cash balances for each exit scenario and exit date and estimated the probability and timing of each potential liquidity event based on management s best estimate taking into consideration all available information as of the valuation date, including the stage of clinical development of our lead drug candidate, industry clinical success rates, our expected near-term and long-term funding requirements, and an assessment of the current financing and biopharmaceutical industry environments at the time of the valuation.

Our May 2011 valuation utilized probabilities of potential liquidity events including: (i) IPO in February 2012 of 10%, (ii) IPO in February 2013 of 10%, (iii) IPO in September 2013 of 15%, (iv) M&A-low in February 2013 of 15%, (v) M&A-high in February 2013 of 10%, (vi) M&A in September 2013 of 15%, (vii) reverse merger in February 2012 of 5% and (viii) liquidation in February 2013 of 20%. Our December 31, 2011, March 31, 2012 and June 30, 2012 valuations utilized probabilities of potential liquidity events including: (i) IPO in September 2013 of 20%, (ii) M&A-low in December 2013 of 25%, (iii) M&A-high in December 2013 of 10%, (iii) reverse merger in September 2013 of 15% and (iv) liquidation in December 2013 of 30%. Our September 30, 2012 valuation utilized probabilities of potential liquidity events including: (i) IPO in March 2013 of 45%, (ii) IPO in September 2013 of 15%, (iii) M&A in September 2013 of 25% and (iv) liquidation in December 2013 of 15%. Our December 31, 2012 valuation utilized probabilities of potential liquidity events including: (i) IPO in March 2013 of 50%, (ii) IPO in September 2013 of 15%, (iii) M&A in September 2013 of 25% and (iv) liquidation in December 2013 of 10%. Our March 31, 2013 valuation utilized probabilities of potential liquidity events including (i) IPO in May 2013 of 60%, (ii) IPO in October 2013 of 10%, (iii) M&A in October 2013 of 20% and (iv) liquidation in December 2013 of 10%.

We used a 20% weighted average cost of capital for each quarterly valuation in 2011 and 2012 and we used discounts for lack of marketability based on option pricing models utilizing the expected time to liquidity in each scenario. The discounts for lack of marketability used in the valuations at May 2011, December 31, 2011, March 31, 2012, June 30, 2012, September 30, 2012 and December 31, 2012 were 21% 59%, 40%, 35%, 19% 30% and 18% 25%, respectively. During the first quarter of 2013, we reduced our range of discounts for lack of marketability to 5% 15%.

In October 2012 and April 2013, our stockholders approved a 1-for-100 reverse common stock split and a 1-for-24 reverse common stock split, respectively, for which we have retroactively adjusted all of our historical common stock price, share and fair value disclosures including the amounts presented in the table below.

From our August 18, 2011 grant date to our September 30, 2011 grant date we experienced a significant decrease in our common stock valuation primarily as a result of revisions to our planned regulatory approval path

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of our lead drug candidate which extended the expected time until commercialization of such drug candidate. From our September 30, 2011 grant date to our April 24, 2012 grant date the fair value of our common stock remained relatively consistent as we did not have any material operational or development milestones that would cause material changes in our overall enterprise value.

In October 2012, in connection with the 1-for-100 reverse common stock split, the conversion rates of our preferred stock were adjusted to reflect a 1-for-1 (rather than a 1-for-100) conversion rate (except Series D which was adjusted back to its previous 2.21-for-1 conversion rate). These conversion rate adjustments were unrelated to the dilution protections afforded to the holders of our preferred stock under our certificate of incorporation. The impact of this conversion rate adjustment further diluted the value held by the common stockholders for shares or rights to purchase shares acquired prior to October 26, 2012, and is reflected on a prospective basis. As a result of this adjustment, the fair value of our common stock decreased from $168.00 per share in April 2012 to $6.00 per share as of December 2012. This decrease reflects the fact that the adjustment to the conversion rates of our preferred stock allocated a greater percentage of our estimated enterprise value to the preferred stockholders and essentially rendered valueless all previously granted common stock instruments.

From December 31, 2012 to our March 31, 2013 valuation date, the fair value of our common stock increased from $6.00 per share to $8.64 per share. The increase in the fair value per share was primarily due to our selection of higher market comparables in our first quarter 2013 valuation, our decision to increase the near-term probability of an IPO and our use of lower discounts for lack of marketability in our valuation models. These changes were due to several key events occurring in the first quarter of 2013, namely our February 2013 meeting with the FDA and the termination of our agreement with Astellas (ending in September 2013). We believe the positive results from our meeting with the FDA served to remove some near-term development risk for quizartinib, and the meeting resulted in our initiation of discussions with the FDA related to the possible acceptance of two novel surrogate endpoints that could support an accelerated approval of quizartinib based on the results of our Phase 2 clinical trials. Negative feedback from the FDA would have been highly detrimental to our near-term business model. In March 2013, we were notified by our partner, Astellas, of its exercise of the termination for convenience clause of our collaboration agreement. As a result, the agreement will terminate effective in September 2013, following which we will own exclusive world-wide rights to quizartinib and any follow-on compounds and will be responsible for all development and commercialization activities and related costs. Although the long-term impact of this termination is not fully known, we have factored the change in status with Astellas into our valuation models and we considered our retention of the exclusive rights to quizartinib when we selected higher market comparables in our first quarter 2013 valuation.

Summary of Stock Option Grants

The following table compares the originally determined value (exercise price) and reassessed value for all option grants from January 1, 2011 to December 31, 2012:

Grant Date	Number of Shares Subject to Options Granted	Exercise Price per Share	Reassessed Estimated Fair Value of Common Stock per Share at Date of Grant	Intrinsic Value per Share at Date of Grant
August 18, 2011	1,768	$ 600.00	$ 576.00	$
September 30, 2011	36	600.00	144.00	
September 30, 2011 (repricing)	1,291	1,680.00	144.00	
January 12, 2012	2,864	600.00	144.00	
April 24, 2012	520	600.00	168.00	
December 13, 2012	1,214,750	6.00	6.00	

We did not grant any stock options during the three months ended March 31, 2013.

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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.

Total stock-based compensation expense included in the consolidated statement of operations was allocated as follows (in thousands):

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
Research and development	$ 224	$ 129	$ 39	$ 111
General and administrative	1,163	612	168	287
Total	$ 1,387	$ 741	$ 207	$ 398

Total stock-based compensation expense related to unvested stock option grants not yet recognized as of March 31, 2013 was approximately $3.6 million and the weighted-average period over which these grants are expected to vest is 3.6 years.

Based on the assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), the intrinsic value of stock options outstanding as of March 31, 2013 would be $9.7 million, of which $0.9 million and $8.8 million would have been related to stock options that were vested and unvested, respectively, at that date.

Equity instruments issued to non-employees are recorded at their fair values and are periodically revalued as the options vest and are recognized as expense over the related service period. No non-employee stock-based compensation expense was recorded for the years ended December 31, 2011 and 2012 and the three months ended March 31, 2013.

Warrant Liabilities

We have issued freestanding warrants to purchase shares of our redeemable convertible preferred stock and common stock. The redeemable convertible preferred stock warrants are exercisable for shares of Series C, Series D and Series D-2 redeemable convertible preferred stock and are classified as liabilities in the accompanying consolidated balance sheets, as the terms for redemption of the underlying security are 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. Our outstanding common stock warrants issued in connection with our Series E financing in 2012 are 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 warrants, except as noted below, 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

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value of the warrant liabilities until: (i) exercise, (ii) expiration of the related warrant, or (iii) upon conversion of the redeemable convertible preferred stock underlying the security into common stock. We do not re-measure the fair value of warrants unless they are required to be accounted for as liabilities. As such, all of our outstanding preferred stock warrants and only the common stock warrants issued in 2012 in connection with our Series E financing are re-measured on a periodic basis. The consummation of this offering will result in the conversion of all classes of our preferred stock into common stock. Upon such conversion of the underlying classes of preferred stock, the preferred stock warrants will be classified as a component of stockholders equity and will no longer be subject to remeasurement.

Redeemable Non-Controlling Interest

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, we adjust 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 is 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. The redeemable non-controlling interest will continue to be measured at fair value until the time at which no Class C, Series D or Series D-2 shares of Ambit Canada are held by GrowthWorks Canadian Fund Ltd., or GrowthWorks, or any other third party, at which time the redeemable non-controlling interest will be reclassified to additional paid-in capital.

Net Operating Loss and Research and Development Tax Credit Carryforwards

As of December 31, 2012, we had federal and California tax net operating loss carryforwards of $151.2 million and $143.1 million, respectively, which begin to expire in 2022 and 2013, respectively, unless previously utilized. As of December 31, 2012, we also had federal and California research and development tax credit carryforwards of $5.0 million and $5.7 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.

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

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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 Three Months Ended March 31, 2012 and 2013

Collaboration Agreement Revenues. We recorded revenues of $5.2 million and $6.6 million for the three months ended March 31, 2012 and 2013, respectively, under our agreement with Astellas. The increase of approximately $1.4 million was primarily due to an increase in license fee amortization revenue being recognized during the three months ended March 31, 2013. In March 2013, we received a notice of termination of the agreement from Astellas, which termination will be effective in September 2013. Accordingly, the remaining deferred revenue related to the upfront license fee is being 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. 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.

Research and Development Expenses. Our research and development expenses were $11.1 million and $9.0 million for the three months ended March 31, 2012 and 2013, respectively. A comparison of research and development expenses by category is as follows (in thousands):

	Three Months Ended March 31,		Increase/ (Decrease)
	2012	2013	
Outside services	$ 8,613	$ 6,708	$ (1,905)
Salaries and personnel	1,660	1,704	44
Facilities and operations	867	593	(274)
Total	$ 11,140	$ 9,005	$ (2,135)

Outside Services. Expenses for outside services, such as for CROs and investigator sites, decreased approximately $1.9 million from $8.6 million for the three months ended March 31, 2012 to $6.7 million for the three months ended March 31, 2013. This decrease was due to lower quizartinib research and development expenses, resulting from a reduction in the number of patients being treated and followed in our Phase 2 clinical trial.

Facilities and Operations. Expenses for facilities and operations decreased approximately $274,000 from $867,000 for the three months ended March 31, 2012 to $593,000 for the three months ended March 31, 2013. The decrease was primarily due to a decrease in our rent expense, as our monthly rent expense for our Sorrento Valley Boulevard facility was reduced to approximately $55,000 per month effective July 2012.

Interest Expense. Interest expense decreased approximately $194,000 from $356,000 for the three months ended March 31, 2012 to $162,000 for the three months ended March 31, 2013. The decrease in interest expense

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was due to the decrease in the principal balance of our outstanding venture loan as we pay down more principal near the maturity of the loan.

Change in Fair Value of Warrant Liabilities. During the three months ended March 31, 2012 and 2013, the change in fair value of the stock warrant liabilities related primarily to increases in the fair value of the underlying preferred or common securities. The change in fair value in 2013 was $3.4 million higher than the change in fair value in 2012 due primarily to the impact of common stock value increases on the 1.1 million common stock warrants issued in October 2012 in connection with our Series E preferred stock financing.

Comparison of the Years Ended December 31, 2011 and 2012

Collaboration Agreement Revenues. We recorded revenues of $23.8 million and $17.6 million for the years ended December 31, 2011 and 2012, 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 $50.7 million and $36.7 million for the years ended December 31, 2011 and 2012, respectively. A comparison of research and development expenses by category is as follows (in thousands):

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

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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.

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 the initial public offering contemplated by this prospectus.

Liquidity and Capital Resources

We have incurred losses since inception and negative cash flows from operating activities for 2011 and 2012 and for the three months ended March 31, 2013. As of March 31, 2013, we had an accumulated deficit of $245.2 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 due October 1, 2013 includes additional interest of 3.0% of the initial loan amount, or $360,000, which is being 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

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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. As of March 31, 2013, the remaining principal balance on the Venture Loans was $3.1 million.

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 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.

The report of our independent registered public accounting firm on our audited consolidated financial statements for the year ended December 31, 2012 includes an explanatory paragraph stating that our recurring losses from operations and working capital deficit raise doubt about our ability to continue as a going concern. If we are unable to obtain additional financing on commercially reasonable terms, our business, financial condition and results of operations will be materially adversely affected and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our financial statements.

We estimate that our net proceeds from this offering and the concurrent private placement will be approximately $82.7 million, based upon an assumed initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and offering expenses payable by us. We expect that the net proceeds from this offering and the concurrent private placement and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our capital requirements for at least the next 12 months.

From our inception through March 31, 2013, we have funded our consolidated operations primarily through the private placements of equity and convertible debt securities and upfront payments 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 March 31, 2013, we had cash and cash equivalents of approximately $8.4 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,		Three Months Ended March 31,	
	2011	2012	2012	2013
Net cash used in operating activities	$ (39,626)	$ (28,772)	$ (5,582)	$ (9,106)
Net cash (used in) provided by investing activities	(153)	46	(2)	(499)
Net cash provided by (used in) financing activities	18,594	29,759	(1,164)	664
Effect of exchange rate changes on cash	(246)	31	36	(135)
Net (decrease) increase in cash and cash equivalents	$ (21,431)	$ 1,064	$ (6,712)	$ (9,076)

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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.

Cash used in operating activities increased $3.5 million from $5.6 million for the three months ended March 31, 2012 to $9.1 million for the three months ended March 31, 2013. Non-cash expenses increased $3.9 million from $622,000 for the three months ended March 31, 2012 to $4.5 million for the three months ended March 31, 2013. Changes in working capital and deferrals provided cash of $1.8 million for the three months ended March 31, 2012, and used cash of $5.3 million for the three months ended March 31, 2013.

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. 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 three months ended March 31, 2012 and 2013, investing activities used cash of $2,000 and $499,000, respectively, primarily due to purchases of property and equipment.

Financing activities in the year ended December 31, 2011 provided net cash of $18.6 million, compared to $29.8 million during the year ended December 31, 2012. 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. 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.

Principal debt payments increased from $3.1 million for the year ended December 31, 2011 to $4.8 million for the year ended December 31, 2012, as principal payments on our venture loan commenced in April 2011.

Financing activities used cash of $1.2 million for the three months ended March 31, 2012 and provided cash of $664,000 for the three months ended March 31, 2013. During the three months ended March 31, 2013, we issued Series E put shares, which provided approximately $2.7 million. Principal debt payments increased from $1.2 million for the three months ended March 31, 2012 to $1.3 million for the three months ended March 31, 2013.

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 share 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.

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The following table summarizes our contractual obligations at December 31, 2012 including interest (in thousands):

	Payments Due by Period				
	Total	Less than 1 Year	1-3 Years	3-5 Years	More than 5 Years
Long-term debt (including interest)	$ 5,024	$ 5,024	$	$	$
Operating lease obligations	3,976	615	1,497	1,339	525
Total	$ 9,000	$ 5,639	$ 1,497	$ 1,339	$ 525

Our commitment for long-term debt relates primarily to the $12.0 million venture loan executed in March 2010, of which $4.3 million was outstanding as of December 31, 2012.

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

We believe that the net proceeds from this offering and the concurrent private placement and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations through at least the next 12 months and will be sufficient to fund the continued development of quizartinib through receipt of topline data from our planned Phase 3 clinical trial in patients with relapsed/refractory AML. 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.

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

- the progress, costs and results of our Phase 2 and Phase 2b clinical trials and our anticipated Phase 3 clinical trial for quizartinib;
- the initiation, progress, timing and results of preclinical studies and clinical trials for any of our other drug candidates;
- the outcome, timing and cost of regulatory approvals;
- 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.

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.

Related Party Transactions

For a description of our related party transactions, see Related Party Transactions.

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Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

Our cash and cash equivalents as of March 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.

Our long-term debt bears interest at a fixed rate and therefore has minimal exposure to changes in interest rates.

Foreign Currency Risk

Our balance sheet as of March 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, 2011 and 2012 and the three months ended March 31, 2013.

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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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 aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, which we formerly referred to as AC220, is a once-daily, orally-administered, potent and selective inhibitor of FMS-like tyrosine kinase 3, or FLT3. Quizartinib is currently in Phase 2b clinical development in patients with relapsed/refractory acute myeloid leukemia, or AML, who express a genetic mutation in FLT3. 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.

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, therefore, proven to be a rich source of targets for drug development with 19 approved drugs in oncology and inflammatory disease since 2001. However, the key technical limitation in the development of drugs that target kinases is the ability to design a drug that selectively inhibits the specific kinase underlying disease while minimizing activity against other kinases, or off-target activity, which can cause undesirable side effects and lead to suboptimal efficacy. 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 chemical library of approximately 8,000 compounds designed to inhibit kinases and expect to continue to leverage this library to develop viable drug candidates in the future. Our current pipeline includes the following:

Quizartinib Our lead drug candidate, quizartinib, is a once-daily, orally-administered, potent and selective inhibitor of FLT3, a validated target in the treatment of AML, and is currently in Phase 2b clinical development. 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. Data from our single-arm, 333 patient Phase 2 clinical trial in relapsed/refractory AML patients was reported at the American Society of Hematology meeting in December 2012. When compared to reported results of clinical trials with other kinase inhibitors with FLT3 activity, quizartinib demonstrated superior single-agent activity in relapsed/refractory AML patients. We are developing a companion diagnostic test with Genoptix Medical Laboratory, a Novartis company, or Genoptix, to identify FLT3-ITD positive patients. We plan to develop quizartinib in other AML therapeutic settings, irrespective of FLT3-ITD status, including use in newly diagnosed AML patients in combination with chemotherapy, or frontline therapy, followed by continuous single-agent maintenance therapy, as well as maintenance following a hematopoietic stem cell transplantation, or an HSCT (commonly referred to as a bone marrow transplant).

AC410 Our second most advanced drug candidate, AC410, is a potent, selective, orally-administered, small molecule inhibitor of JAK2, which has potential utility for the treatment of autoimmune and inflammatory diseases. Signaling through JAK controls the activation, proliferation and survival of various types of immune

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cells, and overactivation of such cells can exacerbate a variety of normal inflammatory processes, resulting in inflammation. 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 have selected AC410 over AC430 and AC409 for further clinical development due to its superior pharmacokinetics as observed in this clinical trial. To our knowledge, AC430 was the first selective JAK2 inhibitor to be advanced into clinical development for inflammatory disease and we believe AC410 may offer distinct benefits in this commercially attractive drug category. We plan to advance AC410 to proof-of-concept clinical trials in one or more autoimmune and inflammatory diseases, independently or in collaboration with a strategic partner.

CSF1R Program We are developing a potent and exquisitely selective small molecule compound, AC708, that inhibits CSF1R and has potential utility in oncology, autoimmune and inflammatory diseases. Signaling through CSF1R controls the activation, proliferation and survival of macrophages, which are key mediators of immune system function, and over-activation of macrophages may result in exacerbation of certain diseases. We have initiated investigational new drug, or IND, -enabling studies with AC708. We plan to further develop this program 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. To support a new drug application, or NDA, pending input from regulatory authorities, we plan to initiate a randomized, comparative Phase 3 clinical trial in relapsed/refractory AML patients with the FLT-ITD mutation by early 2014.

Maximize the therapeutic potential of quizartinib in AML and other hematological disease indications. Beyond our initial focus in relapsed/refractory AML, we plan to develop quizartinib in other AML therapeutic settings, irrespective of FLT3-ITD status, including use in newly diagnosed AML patients in combination with chemotherapy, or frontline, followed by continuous single-agent maintenance therapy as well as maintenance following HSCT.

Maximize strategic value by establishing a commercial capability to market, sell and distribute quizartinib in North America. We plan to build the capabilities to effectively commercialize quizartinib in North America.

Pursue strategic partnerships to accelerate development and expand the commercial opportunity for quizartinib . We plan to retain commercial rights to quizartinib in North America, however, we are considering strategic partners as a means to accelerate the broader clinical development of quizartinib and maximize its therapeutic and market potential. Additionally, we plan to enter into collaborative arrangements for commercialization of quizartinib in markets outside of North America.

Advance the development of our JAK2 and CSF1R programs through a combination internal development and strategic partnerships. We intend to leverage our experience in the discovery and development of kinase inhibitors and may enter into future strategic partnerships for these programs to optimize the therapeutic and commercial potential of future drug candidates from these programs.

Leverage our core competency and proprietary chemical library to continue discovering and developing a broad pipeline of novel drug candidates that inhibit validated kinase targets to address diseases with unmet medical need. We believe that there is significant opportunity in the continued development of drugs that selectively inhibit kinases to address unmet medical needs. We have a proprietary chemical library of approximately 8,000 compounds designed to inhibit kinases. We intend to leverage this library and our expertise and experience to build sustainable value through the discovery, optimization and development of an even broader pipeline of kinase-targeted drug candidates.

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Background

Kinases are a large family of over 500 enzymes, collectively known as the kinome, that function as important mechanisms of 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 cancer, autoimmune and inflammatory diseases. Therefore, kinases have proven to be a rich source of targets for drug development with 19 approved drugs since 2001 in oncology and inflammatory disease.

Aberrant kinase function, caused by mutations or over-expression, underlies many cancer cell processes, making the kinome an important source for therapeutic targets in oncology. Discoveries of specific drivers of disease have led to the development of targeted therapies, or the tailoring of therapies to a particular tumor or disease profile. These therapies, in some cases, have proven to be more efficacious while having fewer side effects than traditional non-targeted therapies, such as chemotherapy, which kill healthy cells along with cancer cells. Examples of successful development of oral targeted kinase inhibitors include Novartis AG s Gleevec® (imatinib), a BCR-ABL kinase inhibitor for the treatment of Philadelphia chromosome positive chronic myelogenous leukemia, and GlaxoSmithKline plc s Tykerb® (lapatinib), a HER2 kinase inhibitor for the treatment of a subset of breast cancer patients over-expressing the HER2 kinase. Further examples of targeted, oral oncology drugs include Pfizer Inc. s Xalkori® (crizotinib) and Bosulif® (bosutinib) and Bristol-Myers Squibb s Sprycel® (dasatinib). We believe that therapies that target specific genetic abnormalities in subsets of cancer patients identified through diagnostic tests will result in streamlined clinical trials, stratified patient populations and improved patient outcomes and will be increasingly important in the continued evolution of the treatment of cancer.

Opportunities for kinase drug development extend beyond oncology. The immune system and inflammatory processes are increasingly understood to play important roles in many disease states, and kinases are key mediators of cellular signaling, activation, proliferation, survival and differentiation of immune and inflammatory cells. Autoimmune and inflammatory diseases are often characterized by an overactive immune system response, which can be controlled through the inhibition of specific kinase signaling pathways. For example, rheumatoid arthritis, or RA, an autoimmune disease, has been the focus of significant effort in the discovery and development of kinase inhibitors, and Pfizer Inc. s Xeljanz® (tofacitinib), targeting the JAK family of kinases, was recently approved for treatment of RA. While there is a large opportunity for the development of kinase inhibitors in autoimmune and inflammatory diseases, the chronic nature of these diseases requires a safety profile to accommodate long term dosing. The safety profile of kinase inhibitors is often predicted by their selectivity profile, which represents their ability to target a single or small number of kinases.

Due to structural similarities among kinases, the key technical challenge in the development of targeted kinase inhibitors is the ability to design a drug that selectively and potently inhibits the specific kinase underlying disease while minimizing activity against other kinases, or off-target activity, which can lead to undesirable side effects and result in suboptimal efficacy. Our core competency is our ability to discover, optimize and develop drug candidates that are highly selective and potent against specific kinases.

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Our Pipeline of Targeted Therapies

We have developed a pipeline of small molecule targeted therapies using our expertise in kinase drug discovery and development. The following table summarizes this pipeline:

Quizartinib an Oral FLT3 Inhibitor for AML

Overview of Quizartinib

Our lead drug candidate, quizartinib, is a once-daily, orally-administered, potent and selective inhibitor of FLT3, a validated target in the treatment of AML, and is currently in Phase 2b clinical development in relapsed/refractory AML patients who express the FLT3-ITD mutation, or FLT3-ITD positive patients. The FLT3-ITD mutation acts like a power switch that causes 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. Since 2009, we have been developing quizartinib with a partner, Astellas Pharma Inc. and Astellas US LLC, collectively Astellas. Our agreement with Astellas will terminate effective in September 2013, following which we will own exclusive worldwide rights to quizartinib and any follow-on compounds and will be responsible for all development and commercialization activities and related costs. We and Astellas are currently working on a transition plan for current and future development activities and do not anticipate that such transition will delay the clinical development activities described in this prospectus.

We initiated a Phase 2 clinical trial of quizartinib in relapsed/refractory AML patients in November 2009 and completed enrollment in November 2011. In this 333 patient single-arm clinical trial, quizartinib demonstrated the ability to significantly reduce the number of blasts in the bone marrow of a substantial number of patients, often for a clinically meaningful duration of time. The reduction of blasts benefited patients who, as a result of responding to quizartinib, may have become eligible for a potentially-curative HSCT which has been shown to prolong survival for these patients. Reduction in bone marrow blasts generally equates to a positive impact on the overall quality of life and the overall survival of patients, irrespective of HSCT eligibility. Based on our clinical trial data and our review of published results from clinical trials of other drug candidates that inhibit FLT3 that are in, or have been in, clinical development for the treatment of AML, we believe that quizartinib has demonstrated superior ability to reduce bone marrow blasts, which we believe is due to quizartinib s unique combination of potency, selectivity and favorable pharmacokinetics. Quizartinib is currently in a Phase 2b clinical trial to identify the optimal dose for our planned Phase 3 clinical trial.

We believe there is a significant unmet need for more effective treatment of AML, particularly for FLT3-ITD positive patients. Our initial regulatory strategy for quizartinib is focused on relapsed/refractory AML

patients that are FLT3-ITD positive and, in concert with Genoptix, we are developing a companion diagnostic test to identify FLT3-ITD positive patients. We believe this initial indication will require a randomized, comparative Phase 3 clinical trial to support an NDA submission to the FDA and equivalent submissions to foreign regulatory authorities. Based on our clinical trial results to date, we plan to develop quizartinib in other AML patient populations including newly-diagnosed patients, FLT3-ITD negative patients and post-HSCT patients.

Background of Current Treatment for AML

AML is the most common type of acute leukemia in adults and is projected to account for approximately 29% of all new leukemia cases in 2012. 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 13,780 patients will be newly diagnosed with AML in 2012 in the United States and approximately 10,200 are expected to die of the disease in 2012. 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. Treatment decisions for AML are typically based on the patient s age (60 years of age being generally referred to as elderly and used as a treatment indicator), overall health, cytogenetics and FLT3-ITD status. These prognostic factors determine the aggressiveness of the treatment approach given the high toxicity associated with currently approved treatment options for AML. Importantly, the National Comprehensive Cancer Network recommends participation in clinical trials as a treatment option for all AML patients.

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 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 complete platelet recovery, and CRp, which is a complete remission with incomplete platelet recovery. 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.

Newly diagnosed patients that are less than 60 years of age are typically treated with an initial regimen of intense chemotherapy, referred to as induction chemotherapy. Due to the toxicity and significant myelosuppression associated with induction chemotherapy, patients are frequently hospitalized for the duration of induction chemotherapy to reduce the risk of infections and manage such toxicities. Patients that achieve a CR from induction chemotherapy typically have two treatment options, (1) one or more rounds of additional chemotherapy, referred to as consolidation therapy, which is intended to eliminate any remaining cancer cells and maintain a remission for the patient and, if eligible, (2) an HSCT. We estimate, based on public information, information available to us from third-party physician and market surveys and our clinical experience that approximately 70% of AML patients will be treated with induction chemotherapy or a similarly aggressive treatment, as the first line of therapy and that a majority of those treated will achieve a CR from this treatment. We further estimate that approximately 80% of all AML patients will either be refractory to this first line of therapy (ie. not achieve a CR) or will relapse after achieving the CR. For patients who have relapsed or did not respond to one or more rounds of chemotherapy, referred to as refractory, the next treatment option is typically a clinical trial or palliative care.

Given the high toxicity associated with induction chemotherapy, newly diagnosed AML patients over 60 years of age are often not treated with induction chemotherapy and are candidates for participation in clinical trials. Even with hospitalization, the chemotherapy-related mortality rate for elderly patients undergoing induction chemotherapy is as high as 29%. More recently, this elderly patient population has also been treated with hypomethylating agents such as Celgene Corporation s Vidaza® (azacitidine) and Eisai Inc. s Dacogen®

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(decitabine), which are both injectable drugs. While these agents have shown modest activity in patients with AML, neither of these agents have been approved in the United States for the treatment of AML.

While patients with poor cytogenetics or FLT3-ITD mutations are considered to have more aggressive disease and a higher likelihood of relapse, the standard of care for this patient population has been the same induction and consolidation regimens used in patients without these poor prognostic factors. The primary difference in the treatment paradigms for this patient population is that an HSCT is frequently recommended after induction chemotherapy given the high likelihood of relapse. For these patients, the goal of treatment is to use induction chemotherapy to induce remission and bridge the patient to transplant as quickly as possible. Increasingly, following an HSCT, patients have also been treated with hypomethylating or other agents to help maintain the remission. More recently, patients with FLT3-ITD mutations have been treated with non-selective multi-kinase inhibitors approved for other indications that have shown some activity against FLT3. This class of agents represents the first targeted approach to the treatment of AML. While this class has shown promise, most of these agents have either failed in clinical trials or have not been specifically developed for this indication. As a result, today there is no approved targeted therapy for the treatment of AML.

We believe that there is a significant 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.

Role of FLT3 in AML

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. We estimate, based on publicly-available information about the disease and treatment courses, third-party market surveys, our clinical trial experience and an interpolation of data on the relative incidence of AML (irrespective of FLT3-ITD status) among geographies that, approximately 4,800, 5,300 and 1,800 newly-diagnosed AML patients each year in the United States, Europe and Japan, respectively have the FLT3-ITD mutation. The FLT3-ITD mutation results in the constant ligand-independent activation of FLT3, leading to aggressive proliferation of immature, irregular blasts that lack the ability to differentiate into normal blood cells. Physicians, as a standard part of diagnosis, routinely test patients for the FLT3-ITD mutation. 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, as shown in Figure 1 below.

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Figure 1: Overall Survival for Patients with Normal Cytogenetics Stratified by Absence (N=125) or Presence (N=67) of the FLT3-ITD Mutation. ***Adopted from Fröhling et al, Blood. 2002 100: 4372-4380.***

Furthermore, clinical evidence from a pediatric study suggests that in addition to those AML patients who harbor the FLT3-ITD mutation, there are FLT3-ITD negative AML patients who over-express the normal, or wild-type FLT3 receptor. Progression of the disease in these patients is believed to be driven at least in part by an overactive FLT3 signaling pathway, resulting in a similarly aggressive proliferation of blasts. In this study, it was demonstrated that the 25% of FLT3-ITD negative patients with the highest level of FLT3 over-expression had a prognostic outcome that was equally as poor as for those patients with the FLT3-ITD mutation. These data suggest that AML patients with high levels of overexpression may benefit from treatment with a FLT3 inhibitor.

Figure 2: High Normal (Wild-Type) FLT3 Expressers Overall Survival Compared to FLT3-ITD Patients. ***Adopted from Brown et al, Blood (ASH Annual Meeting Abstracts) 2008 112: Abstract 147.***

FLT3 as a Validated Target Quizartinib Opportunity

Several kinase inhibitors with activity against FLT3 have been evaluated as single agents in AML patients, including Pfizer s Sutent® (sunitinib), Cephalon, Inc. s CEP-701(lestaurtinib), Novartis PKC-412 (midostaurin), Millennium Pharmaceuticals, Inc. s MLN-518 (tandutinib), Bayer AG s and Onyx Pharmaceuticals, Inc. s Nexavar® (sorafenib) and ARIAD Pharmaceuticals, Inc. s Iclusig® (ponatinib). The level of bone marrow blast reduction with quizartinib demonstrated in our Phase 2 clinical trial was greater when compared historically to the level of bone marrow blast reduction observed in other Phase 1 and Phase 2 clinical trials primarily in relapsed or refractory AML patients treated with the kinase inhibitors listed above, as shown in Figure 3 below.

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Figure 3: Incidence of Bone Marrow Blast Reduction to <5% Patients in Monotherapy Trials of other Kinase Inhibitors with FLT3 Activity in Relapsed / Refractory AML.

* Adopted from Knapper et al, Expert Opin. Investig. Drugs (2011) 20(10):1377-1395 (for sunitinib, lestaurtinib, midostaurin, tandutinib, sorafenib) Adopted from Talpaz et al, ASCO 2011 (for ponatinib). Blast reduction (<5%) data based on pooled clinical trial results for sorafenib (Phase 1 N=16, 0/16; Phase 1 N=20, 1/20).

In most cases, these kinase inhibitors were not initially developed to be specifically selective for FLT3 as they each target other kinases, in addition to FLT3. We believe that the lack of selectivity and potency in targeting FLT3, combined with their promiscuity with other kinases, may limit the therapeutic activity of these drugs for the treatment of AML. Quizartinib was specifically designed as a selective and potent FLT3 inhibitor for the treatment of AML, which we believe makes it a better treatment alternative for these patients.

Because of the lack of treatment options for this patient population, commercially-available kinase inhibitors, such as sunitnib and sorafenib, are often used off-label despite the low response rate generated by these drugs. PKC-412 (midostaurin) is the only kinase inhibitor listed above currently in a Phase 3 clinical trial for the treatment of newly diagnosed FLT3-ITD positive patients with AML.

We believe that the prior trials with other kinase inhibitors listed above validate the FLT3 target but there is a significant unmet need for an effective, well-tolerated drug that inhibits FLT3 and that can be dosed as a continuous, once-daily, orally-administered treatment. We believe quizartinib has the desirable combination of potency, selectivity, and favorable pharmacokinetics necessary for the continuous FLT3 inhibition to be effective as an oral monotherapy agent. We also believe that the results from our ongoing Phase 2 clinical trial showing effectiveness in reducing bone marrow blasts, ability to facilitate a bridge to an HSCT and the potential for longer overall survival, demonstrate that quizartinib has the potential to be a transformative treatment for AML patients with the FLT3-ITD mutation.

Our Clinical Program for Quizartinib

We initiated our clinical program in 2007 and to date over 400 patients have been treated in our Phase 1 and Phase 2 clinical trials. We plan to initiate a Phase 3 clinical trial in relapsed/refractory AML patients in early 2014, pending input from regulatory authorities.

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Phase 2 Clinical Trial

We initiated our multi-center, open label Phase 2 clinical trial of quizartinib as monotherapy in relapsed/refractory AML patients in November 2009 and completed enrollment in November 2011. This trial was designed to evaluate the efficacy and safety of quizartinib in relapsed/refractory AML patients both with and without the FLT3-ITD mutation. Overall, a total of 333 patients were enrolled at clinical sites in the United States (highest enrolling country), 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 were enrolled in this clinical trial. A total of 84 patients without the FLT3-ITD mutation were enrolled as well as one patient with 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 that we believe can be treated effectively with quizartinib:

- 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. The median age of patients in Cohort 1 was 69 years, with patients up to the age of 86 years enrolled.

- 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. The median age of patients in Cohort 2 was 51, with patients up to the age of 77 years enrolled.

We conducted two analyses of data during the course of this clinical trial. The first analysis was an interim data analysis that occurred when the first 62 patients had received at least one cycle of treatment. We refer to this first portion of the clinical trial as the exploratory stage. The second data analysis was based on the 190 FLT3-ITD positive patients, 80 FLT3-ITD negative patients, and 1 patient with unknown FLT3 status. We refer to this second portion of the clinical trial as the confirmatory stage. The number of patients enrolled in each stage of the trial, with further breakdown by FLT3-ITD status and cohort are set forth in Table A below.

Table A: Number of Patients Enrolled by Stage, Cohort, and FLT3-ITD Status

	Cohort 1		Cohort 2		
	FLT3-ITD positive	**FLT3-ITD negative**	**FLT3-ITD positive**	**FLT3-ITD negative**	**Total**
Exploratory Stage	22	2	36	2	62
Confirmatory Stage	90	43[1]	100	38	271
Total	112	45[1]	136	40	333

[1] includes one patient of unknown FLT3-ITD status

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The co-primary endpoints of this clinical trial were the following measurements of the reduction of bone marrow blasts, as defined in accordance with modified Cheson criteria (2003): (1) the composite complete response rate (CR+CRp+CRi), or CRc; and (2) the complete response rate, or CR. The response criteria for each of CR, CRp and CRi is as follows:

CR	Reduction in bone marrow blasts to < 5% of bone marrow cells with full hematological recovery
CRp	Reduction in bone marrow blasts to < 5% of bone marrow cells with incomplete platelet recovery
CRi	Reduction in bone marrow blasts to < 5% of bone marrow cells with incomplete neutrophil recovery and with or without complete platelet recovery

Secondary endpoints included transplantation rate, (rate for patients for whom treatment with quizartinib enabled them to become eligible for an HSCT, which we sometimes refer to as, bridged to an HSCT), overall survival, duration of response, disease-free survival and overall response rate, which includes partial response rate (reduction in bone marrow blasts to between 5% and £ 25% of bone marrow cells and ³ 50% reduction in the bone marrow blasts from baseline), or PR.

Overall, the data for the primary and secondary endpoints from the confirmatory stage of this Phase 2 clinical trial were consistent with and confirmed the data from the interim data analysis from the exploratory stage. In the discussion of our Phase 2 clinical trial that follows, we have combined the data from the exploratory and confirmatory stages.

Quizartinib in FLT3-ITD positive patients

Our Phase 2 clinical trial demonstrated the following three key clinical benefits (each of which is described in more detail below):

1. 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 were bridged to a potentially curative HSCT; and

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

In addition, nearly one of every five patients treated with quizartinib (irrespective of FLT3-ITD status) remained alive for more than 12 months and such patients are referred to as long term survivors. As of September 2012, approximately half of the long term survivors remained alive and continued to be followed for overall survival.

1. Quizartinib, as a monotherapy, demonstrated a high response rate in relapsed/refractory FLT3-ITD positive patients

The overall CRc rate in FLT3-ITD positive patients in both cohorts of the Phase 2 clinical trial was 50% (125/248 patients) with a median time for such patients to achieve CRc being 4.3 weeks. A majority of the patients achieved a CRi. Of importance, for relapsed/refractory AML patients, the objective of treatment is achieving remission through a reduction in blasts and then, if available, bridge to an HSCT. In addition, for patients who cannot undergo an HSCT, we believe that the high level of blast reduction correlates with improved quality of life especially given that quizartinib is given orally as an outpatient therapy.

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The median duration of CRc for FLT3-ITD positive patients was 12.1 weeks in Cohort 1 and 10.6 weeks in Cohort 2. The duration of CRc was calculated from the time that a patient first achieved a CRc (bone marrow blasts fell below 5%) until the time that such patient relapsed (bone marrow blasts rose above 5%). See Table B below. As is standard for clinical trials in leukemia, for patients where documented relapse does not occur, i.e. in patients who are bridged to an HSCT in remission, the duration of response is censored and those patients who are last known as responders are not counted as an event of relapse. This potentially impacts the duration of response due to censoring at the time of an HSCT, which often occurs very quickly after a response to quizartinib is achieved. Those bridged to an HSCT without a documented relapse are no longer considered at risk for relapse and therefore we cannot determine whether they could have contributed to an overall longer duration of response.

Table B. Clinical Trial AC220-002: Response in FLT3-ITD Positive Patients (Total N = 248)

	Cohort 1	**Cohort 2**
Response	**N = 112**	**N = 136**
To Quizartinib	**n (%)**	**n (%)**
CRc (CR+CRp + CRi)	63 (56.3)	62 (45.6)
CR	3 (2.6)	5 (3.7)
CRp	4 (3.6)	2 (1.5)
CRi	56 (50.0)	55 (40.4)
PR	23 (20.5)	38 (27.9)
Median Duration of CRc, (weeks) [range]	12.1 [0.1-58.9]	10.6 [0.1-102.1+]

+ indicates that a patient is still being censored for response

The mean duration of treatment for FLT3-ITD positive patients achieving either a CRc or PR, referred to as responders, was 21.8 weeks in Cohort 1, and 15.9 weeks in Cohort 2. See Table C below. Similar to duration of response, duration of treatment is potentially impacted by the number of patients bridged to an HSCT, as treatment with quizartinib is discontinued prior to transplantation.

Table C. Clinical Trial AC220-002: Duration of Treatment in FLT3-ITD Positive Patients (Total N = 248)

	Cohort 1				**Cohort 2**			
	N = 112				**N = 136**			
	Treatment Time in Weeks				**Treatment Time in Weeks**			
	Mean	**Median**	**Min**	**Max**	**Mean**	**Median**	**Min**	**Max**
Responders (CRc or PR)	21.8	17.5	2.0	70.6+	15.9	10.0	2.7	108.1+
Non-responders	5.0	4.4	0.1	12.4	6.9	5.0	0.3	23.3

+ indicates that a patient is still receiving treatment with quizartinib

In addition, and importantly, within both cohorts, a total of 131 FLT3-ITD positive patients were refractory to their last prior therapy. Of those, 74% achieved at least a PR (with 48% achieving a CRc) with quizartinib (not shown). This high level of response allowed many patients to be bridged to an HSCT (especially within Cohort 2) who likely would not have been able to receive an HSCT given their high bone marrow blast percentage while on their previous therapy.

2. Quizartinib bridged a substantial number of patients to a potentially curative HSCT

We believe that bridge to an HSCT is an important clinical benefit of quizartinib. An HSCT remains the only recognized potential cure for relapsed/refractory AML patients and therefore bridge 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 therapy do not typically achieve remission from

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additional therapy, very few relapsed/refractory AML patients are eligible for an HSCT. However, we believe that the level of bone marrow blast reduction and duration of response in patients who responded to quizartinib enabled them to be more likely to become candidates for a potentially curative HSCT.

Of the 248 FLT3-ITD positive patients, 11 out of 112 (9.8%) patients in Cohort 1 and 47 out of 136 (34.6%) patients in Cohort 2 (those receiving their third line of therapy or therapy after a prior HSCT) were bridged to an HSCT. Table D outlines the number of patients who were bridged to an HSCT, as well as analyzes overall survival based on whether or not a patient was bridged to an HSCT. The results below demonstrate the numerical improvement in the median overall survival based on whether or not a patient was bridged to an HSCT.

Table D. Overall Survival by an HSCT for FLT3-ITD Positive Patients (Total N=248)

	Cohort 1	**Cohort 2**
	N = 112	**N = 136**
	n (%)	**n (%)**
# (%) Bridged to an HSCT	11 (9.8)	47 (34.6)
Median Overall Survival (OS) (weeks), [range] (n=248)	25.4 [0.4-96.0+]	24.0 [0.7-109.1+]
Median OS if bridged to an HSCT (weeks), [range] (n=58)	32.7 [12.7-93.0+]	34.1 [13.6-109.1+]
Median OS if not bridged to an HSCT (weeks), [range] (n=190)	24.9 [0.4-96.0+]	18.4 [0.7-108.3+]

+ indicates that subjects are still alive

Within Cohort 2, the one-year survival rate for the patients who were bridged to an HSCT following treatment with quizartinib was 36.2% (17 out of 47 patients remained alive over 1 year). See Figure 4 below. In addition, the median overall survival was 34.1 weeks in those who were bridged to a an HSCT compared to a median overall survival of 18.4 weeks in those who did not undergo an HSCT after treatment with quizartinib. We believe that the significantly higher number of patients who bridged to an HSCT in Cohort 2 is due to the fact that patients in Cohort 2 were, on average, younger than patients in Cohort 1 and older patients are less likely to be eligible for an HSCT.

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Figure 4: 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)

Three patients (n=3/47) that received an HSCT and did not achieve a CRc or PR, had overall survival of 18.3, 30, and 49 weeks, respectively.

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

AML is a deadly disease, particularly for FLT3-ITD positive patients. FLT3-ITD positive patients who are relapsed or refractory generally have very poor response rates to additional therapy and poor overall survival.

Distinct Survival Benefit for Cohort 1: 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.

In addition, as shown in Figure 5 below, in patients who were available for response at day 28, if a patient achieved a PR or CRc to quizartinib, the median overall survival was 31.1 weeks compared to a median overall survival of 12.9 weeks in those who did not achieve at least a PR to quizartinib. This figure is 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 5 below.

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Figure 5: 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)

Distinct Survival Benefit for Cohort 2: In Cohort 2 (patients relapsed after two lines of therapy or who were relapsed or refractory to an HSCT), the median overall survival in the FLT3-ITD positive patients, which typically confers a particularly poor survival outcome, was 24.0 weeks (approximately 6.0 months). 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.

Long Term Survivors: 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. 43 of the 59 long-term survivors (72.8%) were FLT3-ITD positive. Of the 59 long-term survivors, 22 patients (37.3%) were from Cohort 1, of which only one (4.5%) underwent an HSCT, and 10/22 (45.5%) remained alive as of September 2012; and 37 patients (62.7%) were from Cohort 2, of which 26/37 (70.3%) underwent an HSCT, and 21/37 (56.8%) remained alive as of September 2012. 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.

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Quizartinib in FLT3-ITD negative patients

While our development plans for quizartinib are currently focused on the treatment of FLT3-ITD positive patients, we did see responses from FLT3-ITD negative patients in our Phase 2 clinical trial, see Table E. A total of 84 FLT3-ITD negative patients were enrolled in the Phase 2 trial and the co-primary endpoint of CRc was achieved in 28/84 (33.3%) of those patients, with a majority of the patients achieving a CRi. The median duration of CRc was 10.8 weeks in Cohort 1 and 7.0 weeks in Cohort 2.

Table E. Clinical Trial AC220-002: Response in FLT3-ITD Negative Patients (Total N = 84)

	Cohort 1	**Cohort 2**
Response	**N = 44**	**N = 40**
To Quizartinib	**n (%)**	**n (%)**
CRc (CR+CRp+CRi)	16(36.4)	12(30.0)
CR	2(4.5)	1(2.5)
CRp	1(2.3)	1(2.5)
CRi	13(29.5)	10(25.0)
PR	4(9.1)	6(15.0)
Median Duration of CRc, (weeks) [range]	10.8[2.0-57.1]	7.0[0.1-8.1]

Table F. Clinical Trial AC220-002: Duration of Treatment in FLT3-ITD Negative Patients (Total N = 84)

	Cohort 1				**Cohort 2**			
	N= 44				**N= 40**			
	Treatment Time in Weeks				**Treatment Time in Weeks**			
	Mean	**Median**	**Min**	**Max**	**Mean**	**Median**	**Min**	**Max**
Responders (CRc or PR)	28.7	24.0	5.9	77.0	10.0	8.6	4.0	21.9
Non-responders	7.1	4.1	1.1	32.6	9.7	6.7	1.0	38.1

We believe that some of the responses seen in the FLT3-ITD negative patients can be attributed to the 10% ITD level cut-off used to identify FLT3-ITD positive patients in our currently validated diagnostic test, which resulted in patients with less than 10% ITD mutation classified as ITD negative. A test with a lower level of ITD expression detection may be able to appropriately identify more patients as FLT3-ITD positive, and we believe that patients with lower ITD expression should respond similarly to those we identified as FLT3-ITD positive for the purposes of this clinical trial. Based on this, additional work is currently ongoing to validate a lower cut-off on the companion diagnostic to better identify the FLT3-ITD positive population. However, the results from this clinical trial also suggest that patients without the FLT3-ITD mutation may also respond to quizartinib and we are evaluating the potential use of quizartinib for that patient population as an opportunity for expansion of the target population for quizartinib in the future.

Similar to the FLT3-ITD positive patients, one of the most important clinical benefits of treatment with quizartinib for patients identified as FLT3-ITD negative in the Phase 2 clinical trial was the ability of quizartinib to bridge patients to an HSCT. One patient (2.3%) in Cohort 1 and 14 patients (35.0%) in Cohort 2 were bridged to an HSCT.

The median overall survival for Cohort 1 was 19.1 weeks and 25.1 weeks for Cohort 2. Additionally, the median overall survival in Cohort 2 for those bridged to an HSCT has yet to be reached compared to the median overall survival of 19.2 weeks for those within Cohort 2 that did not undergo a subsequent HSCT.

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Figure 6: Cohort 2 Overall Survival for FLT3-ITD Negative Patients Who Were Bridged to an HSCT (N=14) Compared to Those Who Were Unable to Receive an HSCT (N=26)

Safety

To date, the clinical development program for quizartinib includes over 400 patients treated in our Phase 1 and Phase 2 clinical trials in relapsed/refractory AML. 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) in the Phase 2 clinical trial included gastrointestinal toxicities, 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). 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.

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Table G : Clinical Trial AC220-002: Treatment Emergent Adverse Events Occurring in ³ 20% of Patients by Maximum Grade (Total N = 333)

Adverse Event	Grade 1/2 n (% of N)	Grade3/4 n (% of N)	Total Patients (1) n (% of N)
Nausea	169 (51)	9 (3)	178 (53)
Febrile neutropenia (2)	NA	137 (41)	139 (42)
Diarrhea	122 (37)	14 (4)	136 (41)
Vomiting	120 (36)	11 (3)	131 (39)
Fatigue	95 (29)	18 (5)	113 (34)
Pyrexia (3)	89 (27)	12 (4)	103 (31)
Anemia	11(3)	87 (26)	98 (29)
Electrocardiogram QT prolonged (4)	63 (19)	35 (11)	98 (29)
Edema peripheral	88 (26)	3 (1)	91 (27)
Decreased appetite	81 (24)	9 (3)	90 (27)
Dysgeusia	78 (23)	0	78 (23)
Constipation	68 (20)	2 (1)	71 (21)

Note: Patients are counted once only for each adverse event based on the maximum grade experienced for that event.
(1) Totals may exceed sums of columns due to reporting of adverse events without an associated Grade.
(2) Febrile neutropenia cannot be reported as Grade 1 or 2.
(3) One case of Grade 5 pyrexia and pancytopenia (abnormally low reduction in all blood cells produced by bone marrow) was reported.
(4) All but one case of Grade 3/4 electrocardiogram QT prolongation was Grade 3.

The initial dose of quizartinib in our Phase 2 clinical trial was 200mg/day, the maximum tolerated dose determined in our Phase 1 clinical trial. After observing asymptomatic Grade 3 QT prolongation in 35% of the first 17 patients who were dosed at 200 mg once daily on a continuous basis in the Phase 2 clinical trial, we reduced the dose to 135 mg/day and 90 mg/day for males and females, respectively. This dose reduction was applied to all subsequent patients and successfully decreased the incidence of asymptomatic Grade 3 QT prolongation in these subsequent patients to 16% (higher than the investigator-reported number in Table G, as a result of an independent review of patient electrocardiograms). The different doses for male and female patients are due to the fact that QT prolongation was also observed at a higher rate in females compared to males, consistent with supporting literature which indicates that women have a higher prevalence of QT prolongation in general than men for many hypothesized factors.

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 patient taking quizartinib with multiple concomitant medications in our Phase 2 clinical trial. This event resolved after quizartinib discontinuation.

Ongoing Phase 2b Clinical Trial in FLT3-ITD positive relapsed/refractory AML

We and Astellas initiated a Phase 2b clinical trial in April 2012 to study the efficacy and safety of two lower doses of quizartinib. From and after the effectiveness of the termination of our collaboration with Astellas in September 2013, we will assume all responsibility for the continuation and completion of this clinical trial. This Phase 2b clinical trial is a randomized, open-label clinical trial of quizartinib monotherapy in FLT3-ITD positive patients with AML who are refractory or relapsed after two prior lines of therapy, with or without a prior HSCT. Patients will be randomized equally between two doses of quizartinib, 30 and 60 mg/day, with male and female patients randomized equally between doses. The proposed doses of quizartinib are based on evidence of efficacy

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and safety in doses studied in our Phase 1 clinical trial of quizartinib in relapsed/refractory AML patients, as well as preclinical data demonstrating FLT3 target suppression at doses as low as 30 mg daily. We believe that reducing the dose may improve safety while maintaining efficacy. The primary endpoints of the Phase 2b clinical trial are CRc rate and rate of Grade 2, 3 or 4 QT prolongation at the different doses of quizartinib. The secondary endpoints include bridge to an HSCT, CR rate, duration of remission, and overall survival. As of April 2013, a total of 76 patients have been enrolled. This clinical trial will enroll within the United States and select European countries including France, Germany, Italy, and the United Kingdom.

Preliminary Review of Patient Records

Upon review of patient records from 34 patients in this clinical trial who had initiated dosing at least 28 days prior to January 28, 2013, we observed a CRc rate of 37.5% (6/16) in the 30 mg cohort and 38.9% (7/18) in the 60 mg cohort. Based on the small number of patients with limited follow-on included in this initial review, we believe that this represents similar response rates when compared to the results from our Phase 2 clinical trial. Additionally, four out of the 16 (25%) patients in the 30 mg cohort, and six of the 18 (33.3%) patients in the 60 mg cohort were bridged to transplant, similar to the rate observed in the Phase 2 clinical trial.

The observed rate of QT prolongation decreased in comparison to the Phase 2 clinical trial in the evaluable population of 39 patients who had at least one post-baseline QTc measurement. The observed rate of Grade 3 QT prolongation was 0% (0/20) in patients dosed at 30 mg, and 5.3% (1/19) in patients dosed at 60 mg. In addition, based on an updated safety review as of March 26, 2013 for adverse events, the other treatment emergent adverse events appear to be similar to what we observed in our Phase 1 and Phase 2 clinical trials.

Table H. Clinical Trial AC220-2004: Preliminary Review of Evaluable Patients (Total N = 34)

Response To Quizartinib	30mg Cohort N = 16 n (%)	60 mg Cohort N = 18 n (%)
CRc (CR + CRp + CRi)	**6 (37.5)**	**7 (38.9)**
CR	0	1 (5.6)
CRp	0	1 (5.6)
CRi	6 (37.5)	5 (27.8)
PR	1 (6.3)	7 (38.9)

Table I: Clinical Trial AC220-2004: Treatment Emergent Adverse Events (All Grades) Occurring in ³ 15% of Patients in Either Dose Group (Total N = 41)

	30mg Cohort N =21			60mg Cohort N =20		
Adverse Event	**Grade 1/2 n (% of N)**	**Grade 3/4 n (% of N)**	**Total Patients n (% of N)**	**Grade 1/2 n (% of N)**	**Grade 3/4 n (% of N)**	**Total Patients n (% of N)**
Nausea	2(10)	0	2(10)	6(30)	1(5)	7(35)
Diarrhea	1(5)	0	1(5)	6(30)	1(5)	7(35)
Febrile neutropenia (1)	NA	3(14)	3(14)	NA	6(30)	6(30)
Fatigue	2(10)	0	2(10)	4(20)	0	4(20)
Abdominal pain	1(5)	0	1(5)	4(20)	0	4(20)
Headache	1(5)	1(5)	2(10)	3(15)	0	3(15)
Vomiting	2(10)	0	2(10)	2(10)	1(5)	3(15)
Pneumonia	0	0	0	2(10)	1(5)	3(15)
Acute renal failure	0	0	0	1(5)	1(5)	3(15)*
Neutropenia	0	0	0	0	3(15)	3(15)
Anemia	1(5)	3(14)	4(19)	0	2(10)	2(10)

* Includes 1 case of Grade 5 acute renal failure that was assessed as not drug related.
(1) Febrile neutropenia cannot be reported as Grade 1 or 2.

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Quizartinib Development Strategy

The initial development strategy for quizartinib in the United States is focused on FLT3-ITD positive patients with relapsed or refractory AML. Pending input from regulatory authorities, we plan to initiate a randomized, comparative Phase 3 clinical trial studying quizartinib as monotherapy versus physician s choice of standard chemotherapy in approximately 350 relapsed/refractory AML patients over the age of 18 who are in first salvage therapy in early 2014. The primary endpoint of this clinical trial will be overall survival and we expect to receive topline data in late 2015. The design of this clinical trial is being finalized, and will be based on the full data from the Phase 2 clinical trial, the ongoing Phase 2b clinical trial and an ongoing drug-drug interaction study as well as input from the FDA and other regulatory authorities. This Phase 3 clinical trial will, if successful, be expected to support marketing approval submissions in the United States, the European Union and potentially other countries. In addition, we have initiated discussions with the FDA related to acceptance of two novel surrogate endpoints that could support an accelerated approval based upon the results of our Phase 2 clinical trials, namely CRc rate and bridge to an HSCT. We plan to continue these discussions with the FDA at an end of Phase 2 meeting anticipated to occur in September 2013. Accelerated approval is uncertain and would depend on both the FDA s acceptance of such novel surrogate endpoints as well as the results of our ongoing Phase 2 clinical trials and other components of our NDA.

Based on the responses seen in patients who were classified as FLT3-ITD negative in our Phase 2 clinical trial we plan to evaluate the potential use of quizartinib for that patient population as an opportunity for label expansion following potential approval of quizartinib for the treatment of FLT3-ITD positive patients and also plan to develop quizartinib in other AML therapeutic settings, including in combination with chemotherapy for frontline therapy and as a maintenance therapy following an HSCT and in multiple other hematologic disease indications.

In addition to AML, quizartinib is being evaluated in combination with standard chemotherapeutic agents such as cytarabine and azacitidine in investigator sponsored studies for the treatment of high risk myelodysplastic syndrome, or MDS. The results of these studies will contribute to the future life cycle plan for quizartinib.

Other Ongoing Clinical Trials for Quizartinib

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

In November 2011, we and Astellas initiated 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. We believe quizartinib s tolerability profile allows the possibility of dosing in combination with chemotherapy for the treatment of newly diagnosed AML patients. We also believe that the use of quizartinib in these patient populations will increase the overall CR rate and increase the durability of that response. From and after the effectiveness of the termination of our collaboration with Astellas in September 2013, we will assume all responsibility for the continuation and completion of this clinical trial.

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

In June 2012, we and Astellas initiated a Phase 1 dose-escalating clinical trial to evaluate quizartinib as a maintenance therapy for patients, irrespective of FLT3-ITD status, with AML 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 maintenance therapy with a goal of increasing the duration of remission. From and after the effectiveness of the termination of our collaboration with Astellas in September 2013, we will assume all responsibility for the continuation and completion of this clinical trial.

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Ongoing Investigator-Sponsored Phase 1 Clinical Trial

In addition to the clinical development plan for the treatment of adult patients, in August 2011, we initiated a Phase 1 clinical trial of quizartinib in pediatric patients with either relapsed acute lymphoblastic leukemia, or ALL, or relapsed AML. This clinical trial is ongoing and is being sponsored by the Therapeutic Advances in Childhood Leukemia & Lymphoma Cooperative Group. Currently there are 14 patients enrolled and this clinical trial is expected to enroll no more than 39 pediatric patients. The maximum tolerated dose in this clinical trial will help determine the dose for a planned follow-on Phase 2 clinical trial in pediatric patients to help fulfill the pediatric investigational plan requirement by the European regulatory authorities for marketing approval of quizartinib in Europe.

Completed Phase 1 Clinical Trial in Relapsed/Refractory AML

In May 2012, we concluded an open-label Phase 1 dose-escalation clinical trial in which we evaluated quizartinib as monotherapy in 76 relapsed/refractory AML patients (irrespective of FLT3-ITD status). The primary objectives of this clinical trial were to determine the safety and tolerability, including dose limiting toxicity, of quizartinib. The patients in this clinical trial had undergone a median of three prior treatment regimens. The maximum tolerated dose, with continuous dosing, was determined to be 200 mg/day and the dose limiting toxicity was Grade 3 asymptomatic QT prolongation. Across all dose groups studied in this Phase 1 clinical trial, the overall response rate was 23/76 (30%) with 10 subjects achieving a CRc and 13 subjects achieving a PR. Of the 10 subjects with CRc, two achieved a CR and three achieved a CRp with quizartinib.

Completed Bio-availability Study

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 equivalent bioavailability between the liquid form and the tablet. We anticipate incorporating the tablet in future clinical development, including our planned Phase 3 clinical trial, subject to guidance from the FDA. 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.

Companion Diagnostic

In May 2003, Murphy et al. first published on the development of a routine PCR-based assay to determine the presence or absence of FLT3-ITD mutations in a patient s blood or bone marrow sample. Over the past decade this test has been adopted as a routine component of AML genetic testing laboratories in the United States certified under the federal Clinical Laboratory Improvement Amendments of 1988, or CLIA, and at similarly accredited laboratories across the globe. We believe this assay will help identify patients who are most likely to benefit from quizartinib, and have included this assay as part of the screening criteria for our clinical trials. We expect that regulatory approval of quizartinib will require FDA approval of this FLT3-ITD assay in the form of a companion diagnostic test that has been validated for accuracy, precision and reproducibility. We have partnered with Genoptix to develop a validated companion diagnostic for use in our clinical trials and to prepare and submit a premarket approval application, or PMA, for this companion diagnostic to the FDA in connection with our NDA submission. To support this effort, in partnership with Genoptix, we have had multiple discussions with the Center for Drug Evaluation and Research, or CDER, and the Center for Devices and Radiological Health, or CDRH, at the FDA to collect guidance and feedback on our validation plan for the FLT3-ITD companion diagnostic for quizartinib.

AC410: JAK2-Kinase Specific Inhibitor for Inflammatory Diseases

Overview

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.

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The JAK family comprises of 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. The class of drugs includes two marketed products: Ruxolitinib, a JAK1/2 inhibitor for myelofibrosis (approved in November 2011 and marketed as Jakafi® by Incyte Corporation and Novartis); and tofacitinib, a pan-JAK inhibitor for RA (approved in November 2012 and marketed as Xeljanz® by Pfizer).

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.

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.

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. To our knowledge, AC430 was the first selective JAK2 inhibitor to be advanced into clinical development for inflammatory disease and we believe AC410 may offer distinct benefits in this commercially attractive drug category.

Opportunity for Ambit

Inflammatory diseases are complex and heterogeneous, involving numerous and varying cytokines, and include RA, psoriasis, asthma, multiple sclerosis and Crohn s Disease. Current standards of care include biologic therapies that are directed against a single cytokine or drug target and are administered via infusion or subcutaneous injection. 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.

Autoimmune Disease

The cytokines IL-12 and IL-23 are implicated as central mediators in the development of autoimmune disease due to their key roles in induction of Th1 and Th17 immune responses, respectively, and therapeutic monoclonal antibodies directed against these cytokines, such as Janssen Biotech Inc. s Stelara(ustekinumab), have been shown to be safe and effective in the treatment of psoriasis, and investigation in other autoimmune diseases is ongoing. The cytokine IL-6 is implicated as one of the most important mediators in the acute phase inflammatory response, and therapeutic antibodies directed against this cytokine, such as Genentech s Actemra(tocilizumab), have been shown to be safe and effective in the treatment of rheumatoid arthritis, and investigation in other autoimmune diseases is ongoing. Combined, this data suggests that a small molecule JAK2 inhibitor that blocks IL-6, IL-12, and IL-23 may be able to provide therapeutic benefit in autoimmune and inflammatory diseases such as psoriasis and rheumatoid arthritis.

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Allergic Disease

The cytokine IL-5 has been implicated in the proliferation, survival and trafficking of eosinophils, which play a role in allergic diseases such as asthma. One investigational monoclonal antibody that is directed against this cytokine, GlaxoSmithKline s mepoluzimab, has demonstrated a signification reduction in asthma exacerbations and the reliance on corticosteroids over a twelve month period in a subpopulation of severe asthmatics, and other IL-5 antibodies have also signaled the potential for therapeutic benefit in severe allergic asthma. The cytokine GM-CSF plays an important role in the differentiation, proliferation and survival of granulocytes (e.g. eosinophils, neutrophils and macrophages), and investigational agents appear to have activity in both allergic and non-allergic asthma. The cytokine IL-13 is believed to be a central mediator in allergen-induced asthma, and several monoclonal antibodies have shown activity in investigational clinical studies, particularly in patients with elevated periostin levels. Combined, this data suggests that a small molecule JAK2 inhibitor that blocks IL-5, IL-13, and GM-CSF may be able to provide therapeutic benefit in allergic diseases such as asthma.

AC410 / AC430 Development Summary

AC430 Preclinical Development

We have completed preclinical development and filed an IND for AC430, a potent and selective JAK2 inhibitor. Characterization of AC430 in *in vitro* and *in vivo* studies has demonstrated potent inhibition of JAK2 and the inhibition of signaling of JAK2-mediated cytokines. AC430 demonstrated robust and consistent efficacy in preclinical animal models of arthritic, respiratory, and central nervous system inflammation. IND-enabling toxicology studies have shown AC430 to be very well tolerated in rats and monkeys at exposures significantly higher than what was required to demonstrate cytokine inhibition in the Phase 1 clinical trial.

Selection of AC410

Our initial JAK2 drug candidate, AC430, is a racemic mixture 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 demonstrated potent anti-inflammatory effect in a preclinical animal model of arthritic inflammation and was 40-, 150- and 20- fold more potent for JAK2 activity compared to JAK1, JAK3 and TYK2, respectively, in cellular assays. The FDA has indicated that we can continue the clinical development of AC410 under our IND for AC430 without a need to repeat preclinical toxicology studies completed with AC430, contingent on review by the FDA of the manufacturing process for AC410.

AC430 Phase 1 Clinical Development

We completed enrollment in our dose-ranging Phase 1 clinical trial of AC430 in healthy volunteers to assess the safety, pharmacokinetics and pharmacodynamic effects of AC430. The Phase 1 clinical trial evaluated 84 subjects up to 14 days of continuous dosing. Both once-a-day dosing and twice-a-day dosing regimens of AC430 were evaluated in the multiple ascending dose component of the clinical trial. AC430 was well tolerated and adverse events were all mild-to-moderate, and the most common included dysguesia (effect on sense of taste), gastrointestinal toxicities, headache and fatigue. There were no serious adverse events and we did not observe thrombocytopenia (platelet decrease), neutropenia (neutrophil decrease), anemia (red blood cell decrease), or dyslipidemia (LDL increase), which have been observed in clinical trials of other JAK inhibitors. Although the dosing in this study was of insufficient duration to conclusively determine an absence of these effects, we believe these early signals may translate into potential differentiation in the safety profile of AC410 compared to other JAK inhibitors. Additionally, in pharmacodynamic/biomarker assays, AC430 demonstrated significant and dose-dependent inhibition of JAK2 specific cytokine signaling, at well-tolerated doses. Based on our preclinical and clinical data we believe that AC410 will have a substantially similar safety and activity profile to AC430. Further, we believe the safety and activity demonstrated in the Phase 1 clinical trial data supports further development of AC410 as a once-daily oral therapy for autoimmune and inflammatory diseases.

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AC410 Clinical Development Strategy

We plan to advance AC410 to proof-of-concept clinical trials in one or more autoimmune and inflammatory diseases, independently or in collaboration with a strategic partner.

CSF1R Program: Selective Kinase Inhibitors for Oncology and Inflammation

Overview

We are developing a potent and exquisitely selective small molecule compound, AC708, that inhibits CSF1R and has potential utility in oncology, autoimmune and inflammatory diseases.

Signaling through CSF1R controls the activation, proliferation and survival of macrophages, which are key mediators of immune system function, and over-activation of macrophages may result in exacerbation of certain diseases.

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 can release a wide range of growth factors, enzymes, cytokines, and other inflammatory mediators, which promote angiogenesis (blood vessel formation in tumors), survival and metastasis of tumors and may confer resistance to standard oncology therapies. Cancer cells secrete a variety of growth factors, including CSF-1 (a cytokine that signals through CSF1R), which macrophages require to survive, thereby establishing a co-dependence between macrophages and tumor cells. We believe that a drug that inhibits CSF1R could interrupt the co-dependence between TAMs and tumors, and could be complementary and augment existing standards of care, thereby improving the treatment of cancer.

Macrophages are also a key cellular mediator in the secretion of cytokines and growth factors that can promote inflammation. Over-activation of macrophages is believed to play a role in conditions such as RA, inflammatory bowel diseases, lupus nephritis, atherosclerosis, and even conditions such as obesity. CSF1R plays a central role in the recruitment, activation and survival of macrophages and, as such, CSF1R also represents an attractive target for the treatment of numerous autoimmune and inflammatory diseases.

We are currently conducting IND-enabling studies with AC708.

Opportunity for Ambit

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. The underlying treatment hypothesis requires chronic dosing, which necessitates exceptionally well tolerated compounds. 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 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.

CSF1R Program Pre-clinical Development Summary

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. Initial toxicology studies have shown AC708 to be generally well

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tolerated in rats at exposures significantly higher than what we believe will be necessary to achieve a therapeutic response in patients. Our preclinical studies in oncology and non-oncology disease models are ongoing.

CSF1R Program Development Strategy

We initiated IND-enabling studies in 2013 with AC708. 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 that inhibits the BRAF kinase, discovered by us that 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.

The BRAF gene is a key component of a pathway involved in normal cell growth and survival. The mutations of the BRAF gene are believed to be drivers of disease, including forms of melanoma, thyroid, colon, ovarian, and lung cancers, and can lead to uncontrolled cell growth and disease progression. BRAF mutations have been identified in approximately seven percent of all cancers and we believe that it represents a promising target for pharmacological intervention. 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.

CEP-32496 has been shown to be potent and sustains anti-tumor activity in mouse xenograft models of melanoma and colon carcinoma. CEP-32496 possesses attractive pharmacokinetic properties upon oral administration and compares favorably with respect to other BRAF kinase inhibitors. Teva submitted an IND for CEP-32496 in oncology in the second quarter of 2012 and preparations for initiation of clinical development are ongoing.

Further Kinase Drug Discovery Opportunities

Our approach to kinase drug discovery is chemistry-centric, focusing on biological targets where we have compounds which have demonstrated selective activity for such target. We believe that it is easier to increase the potency of a selective compound than to increase the selectivity of a potent compound. Throughout our discovery process, from hit identification through lead optimization, our scientists screen every compound synthesized against a comprehensive panel of kinases, ensuring that selectivity is a key driver for optimization along with the standard parameters such as potency and pharmacokinetics. Lack of selectivity significantly increases development risk as off-target toxicities can quickly outweigh clinical benefit. Therefore, we believe our approach reduces development risk and creates the potential for best-in-class compounds.

Our proprietary, kinase-focused chemical library has been developed internally over years of discovery efforts and currently contains approximately 8,000 compounds that are fully-annotated against a comprehensive kinase panel. This library represents a wealth of potential opportunities to rapidly initiate novel kinase inhibitor programs beyond our existing pipeline of drug candidates with highly potent and selective leads. Our collective experience in kinase discovery represents a core expertise that, together with our fully-annotated chemical library, provides the potential for sustainable pipeline expansion.

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Our Strategic Alliances and Collaboration Agreements

Our Collaboration with Astellas

In December 2009, we entered into an agreement with Astellas to jointly research, develop and commercialize certain FLT3 kinase inhibitors in oncology and non-oncology indications. Under the agreement, we granted Astellas an exclusive, worldwide license, with limited rights to sublicense, to develop, commercialize and otherwise exploit quizartinib and certain metabolites and derivatives of those compounds. In March 2013, we received a notice of termination of the agreement from Astellas, which termination will be effective in September 2013.

Pursuant to the agreement, in December 2009, Astellas paid us an upfront, non-refundable fee of $40.0 million. We and Astellas share 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. Astellas is responsible for research and development costs for quizartinib in other geographic markets through September 2013, the effective date of the termination. Following the effective date of the termination, the license and rights granted to Astellas terminate and we will own all rights to quizartinib and any follow-on compounds. We will thereafter be responsible for all world-wide development and commercialization activities and related costs. As contemplated by the agreement, we expect to enter into a transition agreement with Astellas before the effective date of the termination, which agreement is expected to cover, among other things, the transition of the execution and management of ongoing clinical trials and the transfer of data, materials and other related information.

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 received two milestone payments totaling $3.0 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 $47.5 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 believe the FDA approval of this test will satisfy the FDA s requirement that a companion diagnostic test be approved 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. A large 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. 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 . We have developed and continue to develop a patent portfolio around our lead drug candidate quizartinib. A composition of matter patent application covering the small molecule drug quizartinib (and a chemical genus to which quizartinib belongs) issued in the United States as U.S. Patent 7,820,657 and corresponding foreign patents have issued in China, Hong Kong, Japan, Malaysia, Mexico, New Zealand, Russia, Singapore and South Africa, while corresponding foreign patent applications remain pending in Argentina, Australia, Brazil, Canada, Europe, India, Israel, South Korea, Norway, the Philippines, and Taiwan. The U.S. composition of matter patent covering quizartinib will expire in 2028 if we continue to pay the maintenance fees and annuities when due, 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 pending applications that cover stable crystalline forms of quizartinib, metabolites of quizartinib, formulations of quizartinib, methods of manufacturing quizartinib and various therapeutic uses of quizartinib. Collectively, these patents, if they issue, would have patent expirations ranging from

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2028 to 2030 if we continue to pay the maintenance fees and annuities when due, not including any possible additional terms for patent term adjustments or patent term extensions. We do not know if any patent will issue from any of these applications and, if any issue, we do not know whether the issued patents will provide significant proprietary protection or commercial advantage against our competitors or generics. Even if they issue, our patents may be circumvented, challenged, opposed and found to be invalid or unenforceable. We also have a composition of matter patent that contains a chemical genus claim covering AC430 and its enantiomers AC409 and AC410 that issued in the United States as U.S. 8,349,851 and we have corresponding patent applications in the following foreign countries: Argentina, Australia, Brazil, Canada, China, Europe, Hong Kong, India, Indonesia, Israel, Japan, South Korea, Malaysia, Mexico, New Zealand, the Philippines, Russia, Singapore, South Africa and Taiwan. We also filed a composition of matter application that is specific to AC410 (one of the enantiomeric forms, or mirror image forms, of AC430), under the Patent Co-operation Treaty, or PCT, in the U.S. and in Argentina and Taiwan, which are not signatories to the PCT. We have also filed composition of matter patent applications that cover a chemical genus encompassing our most advanced CSF1R candidate, AC708, under the PCT and also in the U.S., Argentina and Taiwan. We have filed patent applications covering solid forms of AC430 and AC410 in the United States. We intend to file additional U.S. and foreign applications based on our ongoing research programs directed to formulations, therapeutic uses, combination therapies and methods of manufacture, to the extent they are discovered or invented.

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, technology 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 companies with whom we share proprietary and 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.

Furthermore, 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.

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 be necessary, we cannot predict whether we or our partners would be able to obtain such a license, 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 if we are sued for infringement. We would vigorously defend ourselves, but we cannot predict whether the patents would be found valid, enforceable or infringed.

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.

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

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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 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

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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, 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 or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution,

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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.

Clinical trials involve the administration of the drug candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor s control, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. 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

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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.

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

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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, 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.

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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 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

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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 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. Endpoints such as CRc, CRi and bridge to HSCT are not currently recognized by the FDA as acceptable surrogate endpoints. We believe the eligibility of quizartinib for consideration for accelerated approval will require acceptance by the FDA of new surrogate endpoints, such as CRc and bridge to HSCT, and will depend on a review of the data from our ongoing Phase 2 clinical trials. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. 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

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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 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

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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 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.

Other 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

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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 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.

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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.

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

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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 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 March 31, 2013, we employed 45 employees, 41 of whom are full-time, 13 of whom hold Ph.D. or M.D. degrees, 31 of whom were engaged in research and development activities and 14 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.

Facilities

We lease approximately 19,440 square feet of space for our headquarters in San Diego, California under an agreement that expires in September 2018. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

Legal Proceedings

We are currently not a party to any material legal proceedings.

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MANAGEMENT

Executive Officers and Directors

The following table sets forth information regarding our executive officers and directors as of April 30, 2013:

Name	Age	Position
Michael A. Martino.	57	President, Chief Executive Officer and Director
Alan Fuhrman	56	Chief Financial Officer
Athena Countouriotis, M.D.	41	Chief Medical Officer
Faheem Hasnain	54	Chairman of the Board of Directors
David Bonita, M.D.	37	Director
Steven A. Elms	49	Director
Standish M. Fleming	66	Director
Allan P. Marchington, Ph.D.	46	Director
David R. Parkinson, M.D.	62	Director
Isai Peimer	35	Director
Joseph Regan	45	Director

Executive Officers

Michael A. Martino. Mr. Martino has served as our President and Chief Executive Officer and as one of our directors since November 2011. From March 2010 until November 2011, Mr. Martino held multiple positions with CareFusion Corporation, a publicly-traded healthcare company, including senior vice president and general manager of diagnostics and senior vice president of innovation, business development and strategy. From January 2009 to March 2010, Mr. Martino was president and chief executive officer of Arzeda Corp., a privately-held enzyme design and development company that he co-founded and remains on the board of directors. From September 1998 to August 2008, Mr. Martino served as president and chief executive officer of Sonus Pharmaceuticals, Inc., a publicly-traded pharmaceutical development company that merged with Oncogenex Pharmaceuticals, Inc. in August 2008. Earlier in his career, he held multiple positions during a 17-year tenure at Mallinckrodt, Inc. in strategic planning, business development, marketing and general management. Based on Mr. Martino s management experience and his pharmaceutical industry experience and service as our President and Chief Executive Officer, the board believes Mr. Martino has the appropriate set of skills to serve as a member of our board. Mr. Martino received an M.B.A. from Virginia Tech and a B.A. from Roanoke College.

Alan Fuhrman. Mr. Fuhrman has served as our Chief Financial Officer since October 2010. From November 2008 to September 2010, Mr. Fuhrman served as vice president and chief financial officer of Naviscan, Inc., a privately-held medical imaging company focused on the management of breast cancer. From September 2004 through August 2008, he served as senior vice president and chief financial officer of Sonus Pharmaceuticals, a publicly-traded pharmaceutical development company that merged with Oncogenex Pharmaceuticals in August 2008. Mr. Fuhrman served as president and chief operating officer of Integrex, Inc., a manufacturing services company, from April 2002 until its acquisition in July 2004. From February 1999 until March 2002, he was the chief financial officer at Capital Stream, Inc., a financial services workflow automation company. Mr. Fuhrman received B.S. degrees in both business administration and agricultural economics from Montana State University. Mr. Fuhrman received his Certified Public Accountant Certification from the State of Oregon; however, currently he is not an active CPA.

Athena Countouriotis, M.D. Dr. Countouriotis has served as our Chief Medical Officer since February 2012. From August 2007 to February 2012, Dr. Countouriotis was a clinical leader within the Pfizer Inc. Oncology Business Unit. From October 2005 to August 2007, she was director of oncology global clinical research at Bristol-Myers Squibb Company, a publicly-traded global pharmaceutical company, with responsibility for leading clinical development of Sprycel® in acute lymphoblastic leukemia and chronic myeloid leukemia. Earlier in her career, she held the position as associate medical director at Cell Therapeutics, Inc. a biopharmaceutical company.

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Dr. Countouriotis received a B.S. from the University of California, Los Angeles and an M.D. at Tufts University School of Medicine. She received her initial training in pediatrics at the University of California, Los Angeles and additional training at the Fred Hutchinson Cancer Research Center in the Pediatric Hematology/ Oncology Program.

Non-Employee Directors

Faheem Hasnain. Mr. Hasnain has served as one of our directors since October 2010, and as the Chairman of the Board since November 2010. Mr. Hasnain is the president and chief executive officer of Receptos, Inc., a drug discovery and development company, a position he has held since December 2010. From December 2008 until its acquisition by Abbott Laboratories in April 2010, he was the president and chief executive officer and a director of Facet Biotech Corporation, a publicly-traded, biology-driven antibody company with a focus in oncology and multiple sclerosis. Mr. Hasnain was president, chief executive officer and a director of PDL BioPharma, Inc., a publicly-traded biotechnology company, from October 2008 until Facet Biotech was spun off from PDL BioPharma in December 2008. From October 2004 to September 2008, Mr. Hasnain served at Biogen Idec Inc., a biotechnology company specializing in neurological disorders, autoimmune disorders and cancer, most recently as executive vice president in charge of the oncology/rheumatology strategic business unit. Prior to Biogen Idec, Mr. Hasnain held roles with Bristol-Myers Squibb, where he was president of the oncology therapeutics network, and for 14 years at GlaxoSmithKline plc and its predecessor organizations. Mr. Hasnain has served on the board of directors of Somaxon Pharmaceuticals, Inc., a publicly-traded specialty pharmaceutical company, since September 2010. Based on Mr. Hasnain s management experience and his pharmaceutical industry experience and in-depth understanding of commercialization and corporate development, the board believes Mr. Hasnain has the appropriate set of skills to serve as a member of our board. Mr. Hasnain received a B.H.K. and B.Ed. from the University of Windsor Ontario in Canada.

David Bonita, M.D. Dr. Bonita has served as a one of our directors since October 2012. He is a principal on the private equity team at OrbiMed Advisors LLC, an investment company, a position he has held since June 2004. From 1998 to 1999, Dr. Bonita was a corporate finance analyst in the healthcare investment banking group of Morgan Stanley, an investment firm. From 1997 to 1998, Dr. Bonita was a corporate finance analyst in the healthcare investment banking group of UBS AG, an investment bank. Based on Dr. Bonita s extensive financial services background and experience in the pharmaceutical and healthcare industries, including his service on many biotechnology company boards of directors, the board believes that Dr. Bonita has the appropriate set of skills to serve as a member of our board. Dr. Bonita received an A.B. degree magna cum laude in Biological Sciences from Harvard University and a joint M.D./M.B.A. from Columbia University where he was elected to the Alpha Omega Alpha Medical Honor Society and Beta Gamma Sigma Business Honor Society.

Steven A. Elms. Mr. Elms has served as one of our directors since 2001 and served as the Chairman of the Board from July 2005 to November 2009. He is a managing director of the Perseus-Soros Biopharmaceutical Fund, L.P., or PSBF, and managing partner of Aisling Capital LLC, both venture capital firms. He joined PSBF in 2000 from the life sciences investment banking group of Chase H&Q (formerly Hambrecht and Quist Group Inc.) where he was a principal. Prior to Hambrecht and Quist, Mr. Elms traded mortgage-backed securities at Donaldson, Lufkin & Jenrette Inc. His previous healthcare sector experience includes over two years as a pharmaceutical sales representative for Marion Laboratories Inc. and two years as a consultant for the Wilkerson Group. Mr. Elms has served on the board of directors of Pernix Therapeutics Holdings, Inc., a publicly-traded pharmaceutical company, since July 2011. Mr. Elms served on the board of directors of MAP Pharmaceuticals, Inc., a publicly-traded pharmaceutical company, from June 2004 to February 2011, and has served on the boards of directors of a number of private companies. Based on Mr. Elms extensive financial services background and experience in the pharmaceutical and healthcare industries, including his service on many biotechnology company boards of directors, the board believes Mr. Elms has the appropriate set of skills to serve as a member of our board. Mr. Elms received a B.A. in human biology from Stanford University and an M.B.A. from the Kellogg Graduate School of Management at Northwestern University.

Standish M. Fleming. Mr. Fleming has served as one of our directors since June 2001. He is a managing member at Forward Ventures, a venture capital firm which he co-founded in 1993. Before establishing Forward

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Ventures, he served as the chairman, president and chief executive officer of GeneSys Therapeutics, Inc. (merged with Somatix Therapy Corp. and subsequently acquired by Cell GeneSys, Inc.). In his capacity as a founding managing member of Forward Ventures, Mr. Fleming has served on the board of directors and as the initial president and chief executive officer of numerous pharmaceutical and biotechnology companies. Based on Mr. Fleming s management experience and his service on other boards of directors in the biotechnology and pharmaceutical industries, including his experience in finance, investor relations and operations, the board believes Mr. Fleming has the appropriate set of skills to serve as a member of our board. Mr. Fleming received a B.A. from Amherst College and an M.B.A. from the University of California, Los Angeles Graduate School of Management.

Allan P. Marchington, Ph.D. Dr. Marchington has served as one of our directors since October 2007. He is a partner at Apposite Capital LLP, a venture capital firm, a position he has held since April 2006. From July 2003 to August 2005, he served as an entrepreneur in residence at Abingworth Management, a venture capital firm. From July 2000 to July 2003, he served as senior vice president, at Millennium Pharmaceuticals, Inc. and served as chairman of Millennium Pharmaceuticals Ltd., the European subsidiary of Millennium Pharmaceuticals, Inc. Prior to Millennium, he was principal founder and CEO of Cambridge Combinatorial Ltd., a biotech company which he founded in 1997 and successfully sold to Millennium Pharmaceuticals, Inc. in 2000. Before setting up Cambridge Combinatorial, Dr. Marchington worked for seven years in a range of therapeutic areas at Pfizer. Based on Dr. Marchington s senior positions in the biotechnology and pharmaceutical industries, including management experience as a chief executive officer and his service on other boards of directors in the biotechnology and pharmaceutical industries, and his experience in research and development, the board believes Dr. Marchington s has the appropriate set of skills to serve as a member of our board. Dr. Marchington received a Ph.D. and B.Sc. in chemistry from the University of Liverpool, United Kingdom.

David R. Parkinson, M.D. Dr. Parkinson joined our board of directors in March 2013. He is a venture partner at New Enterprise Associates, a venture capital firm, a position he has held since April 2012. From July 2007 to June 2012, he served as the president and chief executive officer of Nodality, Inc., a private biotechnology company. Prior to Nodality, Dr. Parkinson was senior vice president of Oncology Research and Development at Biogen Idec, a biotechnology company, and previously served as vice president of Oncology Development at Amgen Inc., a biotechnology committee, and vice president of Global Clinical Oncology Development at Novartis Corporation, a pharmaceutical company. Dr. Parkinson worked at the National Cancer Institute from 1990 to 1997, serving as chief of the Investigational Drug Branch, then as action associate director of the Cancer Therapy Evaluation Program. He has also held academic positions at the M.D. Anderson Cancer Center, University of Texas and New England Medical Center at Tufts University School of Medicine. Dr. Parkinson is a past chairman of the FDA s Biologics Advisory Committee. He is a past president of the International Society of Biological Therapy and past editor of the Journal of Immunotherapy. He currently serves on the National Cancer Policy Forum of the Institute of Medicine. He has recently completed a term on the FDA s Science Board as well as a term on the board of directors of the American Association of Cancer Research, or AACR. He continues to serve as Chairman of the AACR Finance Committee. From December 2008 until April 2010, Dr. Parkinson served as a director of Facet Biotech, a publicly-traded biopharmaceuticals company and since May 2010 has served as a director of Threshold Pharmaceuticals, Inc. a publicly-traded biotechnology company. Based on Dr. Parkinson s medical knowledge and experience, especially in the field of oncology, and his industry perspective, the board believes that Dr. Parkinson has the appropriate set of skills to serve as a member of our board. Dr. Parkinson received his M.D. from the University of Toronto Faculty of Medicine in 1977, with Internal Medicine and Hematology/Oncology training in Montreal at McGill University and in Boston at New England Medical Center.

Isai Peimer. Mr. Peimer has served as one of our directors since March 2011. He is a principal at MedImmune Ventures Inc., an investment company, a position he has held since August 2010. From November 2009 to August 2010, he was an associate analyst at AllianceBernstein LP, a global asset management firm. From April 2008 to January 2009, he was a senior associate at Visium Asset Management, LP, a healthcare-focused investment fund. From June 2005 to April 2008, Mr. Peimer worked as an investment banker at J.P. Morgan & Co. and was a management consultant for the pharmaceutical and biotech sectors. Mr. Peimer s career began at Merck & Co., Inc. Based on Mr. Peimer s extensive financial services background and experience in the pharmaceutical and healthcare industries,

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the board believes that Mr. Peimer has the appropriate set of skills to serve as a member of our board. Mr. Peimer received a B.S. in Chemistry summa cum laude from Emory University and an M.B.A. from the Tuck School of Business at Dartmouth.

Joseph Regan. Mr. Regan has served as one of our directors since August 2009. He is vice president of investments at GrowthWorks Capital, Inc., a venture capital firm, a position he has held since 2003. He has served a wide range of roles within portfolio companies including gestational chief executive officer. Additionally he currently serves as president of an early stage seed fund and serves on the board of directors of numerous private companies. Based on Mr. Regan s expertise in strategic growth and his service on the boards of directors in the biotechnology and pharmaceutical industries, the board believes that Mr. Regan has the appropriate set of skills to serve as a member of our board. Mr. Regan received an Honours B.Sc. from the University of Guelph (Distinction) and an M.B.A. from McMaster University.

Board Composition

Our board of directors currently consists of nine members, eight of whom have been determined to be independent within the meaning of SEC rules and regulations and the Nasdaq Marketplace Rules. Effective upon the closing of this offering, we will divide our board of directors into three classes, as follows:

- Class I, which will consist of Dr. Bonita and Messrs. Elms and Regan, and whose term will expire at our annual meeting of stockholders to be held in 2014;
- Class II, which will consist of Messrs. Fleming and Peimer and Dr. Marchington, and whose term will expire at our annual meeting of stockholders to be held in 2015; and
- Class III, which will consist of Dr. Parkinson and Messrs. Hasnain and Martino, and whose term will expire at our annual meeting of stockholders to be held in 2016.

At each annual meeting of stockholders to be held after the initial classification, the successors to directors whose terms then expire will serve until the third annual meeting following their election and until their successors are duly elected and qualified. The authorized number of directors may be changed only by resolution of the board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed between the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of the board of directors may have the effect of delaying or preventing changes in our control or management. Our directors may be removed for cause by the affirmative vote of the holders of at least 66 2/3% of our voting stock.

Board Leadership Structure

Our board of directors has an independent chairman, Faheem Hasnain, who has authority, among other things, to call and preside over board meetings, including meetings of the independent directors, to set meeting agendas and to determine materials to be distributed to the board of directors. Accordingly, the chairman has substantial ability to shape the work of the board of directors. We believe that separation of the positions of chairman and chief executive officer reinforces the independence of the board in its oversight of our business and affairs. In addition, we believe that having an independent board chairman creates an environment that is more conducive to objective evaluation and oversight of management s performance, increasing management accountability and improving the ability of the board of directors to monitor whether management s actions are in the best interests of the company and its stockholders. As a result, we believe that having an independent board chairman can enhance the effectiveness of the board of directors as a whole.

Role of the Board in Risk Oversight

Our audit committee is primarily responsible for overseeing our risk management processes on behalf of the full board of directors. Going forward, we expect that the audit committee will receive reports from management

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at least quarterly regarding our assessment of risks. In addition, the audit committee reports regularly to the full board of directors, which also considers our risk profile. The audit committee and the full board of directors focus on the most significant risks we face and our general risk management strategies. While the board oversees our risk management, company management is responsible for day-to-day risk management processes. Our board of directors expects company management to consider risk and risk management in each business decision, to proactively develop and monitor risk management strategies and processes for day-to-day activities and to effectively implement risk management strategies adopted by the audit committee and the board of directors. We believe this division of responsibilities is the most effective approach for addressing the risks we face and that our board leadership structure, which also emphasizes the independence of the board in its oversight of our business and affairs, supports this approach.

Board Committees and Independence

Rule 5605 of the Nasdaq Marketplace Rules requires a majority of a listed company s board of directors to be comprised of independent directors within one year of listing. In addition, Nasdaq Marketplace Rules require that, subject to specified exceptions, each member of a listed company s audit, compensation and nominating and governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended. Under Rule 5605(a)(2), a director will only qualify as an independent director if, in the opinion of our board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee: (1) accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries; or (2) be an affiliated person of the listed company or any of its subsidiaries.

In February 2013 and March 2013, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his background, employment and affiliations, including family relationships, our board of directors has determined that none of our directors other than Mr. Martino has a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of our directors other than Mr. Martino is independent as that term is defined under Rule 5605(a)(2) of the Nasdaq Marketplace Rules. Our board of directors also determined that Messrs. Fleming, Peimer and Regan, who comprise our audit committee, Dr. Marchington and Messrs. Elms and Hasnain, who comprise our compensation committee, and Messrs. Hasnain and Peimer and Dr. Bonita, who comprise our nominating and governance committee, satisfy the independence standards for such committees established by the SEC. Each of the members of our compensation committee of the board is an outside director for purposes of Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code, and a non-employee director for purposes of Rule 16b-3 under the Securities Exchange Act of 1934, as amended. In making such determination, the board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances the board of directors deemed relevant in determining independence, including the beneficial ownership of our capital stock by each non-employee director. Currently, our board of directors has determined that all current members of the audit committee satisfy the independence requirements for service on the audit committee.

Board Committees

Our board of directors has established an audit committee, a compensation committee and a nominating and governance committee. The composition of each committee set forth below will be effective upon the closing of this offering. Each committee will operate under a charter approved by our board. Following this offering, copies of each committee s charter will be posted on the Corporate Governance section of our website, www.ambitbio.com.

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Audit Committee

Our audit committee consists of Messrs. Fleming, Peimer and Regan, each of whom is a non-employee director of our board of directors. Mr. Fleming serves as the chair of our audit committee. The functions of this committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- reviewing our annual and quarterly financial statements and reports and discussing the statements and reports with our independent auditors and management;
- reviewing with our independent auditors and management significant issues that arise regarding accounting principles and financial statement presentation, and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our auditors any earnings announcements and other public announcements regarding material developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- preparing the report that the SEC requires in our annual proxy statement;
- reviewing and providing oversight with respect to any related party transactions and monitoring compliance with our code of ethics;
- reviewing our investment policy on a periodic basis; and
- reviewing and evaluating, at least annually, the performance of the audit committee, including compliance of the audit committee with its charter.

Our board of directors has determined that each member of the audit committee meets the financial literacy requirements under Nasdaq Marketplace Rules and that Mr. Fleming qualifies as an audit committee financial expert within the meaning of SEC rules and regulations. In making its determination that Mr. Fleming qualifies as an audit committee financial expert, our board has considered the formal education and nature and scope of Mr. Fleming s previous experience, coupled with past and present service on various audit committees. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

Compensation Committee

Our compensation committee consists of Dr. Marchington and Messrs. Elms and Hasnain. Dr. Marchington serves as the chair of our compensation committee. The functions of this committee include, among other things:

reviewing and recommending to our board of directors the compensation and other terms of employment of our executive officers;

reviewing and recommending to our board of directors performance goals and objectives relevant to the compensation of our executive officers;

evaluating and approving the equity incentive plans, compensation plans and similar programs advisable for us, as well as modification or termination of existing plans and programs;

evaluating and recommending to our board of directors the type and amount of compensation to be paid or awarded to board members;

administering our equity incentive plans;

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establishing policies with respect to equity compensation arrangements;

reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;

reviewing and recommending to our board of directors the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;

reviewing with management our disclosures under the caption Compensation Discussion and Analysis and recommending to the full board its inclusion in our periodic reports to be filed with the SEC;

preparing the report that the SEC requires in our annual proxy statement;

reviewing the adequacy of our compensation committee charter on a periodic basis; and

reviewing and evaluating, at least annually, the performance of the compensation committee.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee consists of Messrs. Hasnain and Peimer and Dr. Bonita. Mr. Hasnain serves as the chair of our nominating and corporate governance committee. The functions of this committee include, among other things:

identifying, reviewing and evaluating candidates to serve on our board of directors;

determining the minimum qualifications for service on our board of directors;

evaluating director performance on the board and applicable committees of the board;

interviewing, evaluating, nominating and recommending individuals for membership on our board of directors;

considering nominations by stockholders of candidates for election to our board;

considering and assessing the independence of members of our board of directors;

developing, as appropriate, a set of corporate governance principles, and reviewing and recommending to our board of directors any changes to such principles;

periodically reviewing our policy statements to determine their adherence to our code of business conduct and ethics and considering any request by our directors or executive officers for a waiver from such code;

reviewing, periodically, the adequacy of its charter; and

evaluating, at least annually, the performance of the nominating and corporate governance committee.

Compensation Committee Interlocks and Insider Participation

No member of our compensation committee has ever been an executive officer or employee of ours. None of our officers currently serves, or has served during the last completed fiscal year, on the compensation committee or board of directors of any other entity that has one or more officers serving as a member of our board of directors or compensation committee. Prior to establishing the compensation committee, our full board of directors made decisions relating to compensation of our officers.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or person performing similar functions. Following this offering, a current copy of the code will be posted on the Corporate Governance section of our website, www.ambitbio.com.

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EXECUTIVE AND DIRECTOR COMPENSATION

Summary Compensation Table for the Year ended December 31, 2012

The following table provides information regarding the compensation earned during the last two completed fiscal years by our (1) principal executive officer and (2) our next two highest compensated executive officers other than the principal executive officer, who we collectively refer to as our named executive officers elsewhere in this prospectus.

Name and Principal Position	Year	Salary ($)	Option awards ($)[1]	Non-equity incentive plan compensation ($)[2]	All other compensation ($)[3]	Total ($)
Michael A. Martino[4]	2012	379,647	1,734,430	204,000	4,494	2,322,571
President and Chief Executive Officer	2011	54,087				54,087
Alan Fuhrman	2012	279,812	362,923	87,095	4,343	734,173
Chief Financial Officer	2011	275,000	104,595	49,500	3,288	432,383
Athena Countouriotis, M.D.[5]	2012	329,202	391,882	100,399	2,447	823,930
Chief Medical Officer						

(1) In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during the respective fiscal year computed in accordance with Financial Accounting Standards Board ASC Topic 718, or ASC 718. Assumptions used in the calculation of these amounts are included in Note 7, Stock Based Compensation to our Financial Statements. These amounts do not reflect the actual economic value that will be realized by the named executive officer upon the vesting of the stock options, the exercise of the stock options, or the sale of the common stock underlying such stock options.

(2) Amounts shown represent performance bonuses earned for the respective fiscal year as described in detail in the section below entitled Annual Performance-Based Bonus Opportunity.

(3) Amounts shown for all individuals represent term life insurance and supplemental long-term disability insurance paid by us on behalf of the named executive officers. For more information regarding these benefits, see below under Perquisites, Health, Welfare and Retirements Benefits.

(4) Mr. Martino became our President and Chief Executive Officer in November 2011.

(5) Dr. Countouriotis became our Chief Medical Officer in February 2012.

Base Salary

Base salaries for our executives are initially established through arm s-length negotiation at the time the executive is hired, taking into account such executive s qualifications, experience, prior salary, the scope of his or her responsibilities, and competitive market compensation paid by other companies for similar positions within the industry. Base salaries are reviewed annually, typically in connection with our annual performance review process, and adjusted from time to time to realign salaries with market levels after taking into account individual responsibilities, performance and experience. In making decisions regarding salary increases, we may also draw upon the experience of members of our board of directors with other companies. The compensation committee has not previously applied specific formulas to determine increases, although it has generally awarded increases as a percentage of an executive officer s then-current base salary. This strategy is consistent with our intent of offering base salaries that are cost-effective while remaining competitive.

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Name	2011 Base Salary	2012 Base Salary
Michael A. Martino	$ 375,000	$ 375,000(1)
Alan Fuhrman	275,000	275,000(2)
Athena Countouriotis, M.D.		365,000

(1) Effective as of October 26, 2012, Mr. Martino s base salary was increased to $400,000 in connection with the closing of our Series E preferred stock financing.

(2) In April 2012, Mr. Fuhrman s base salary was increased 3.5% from his 2011 base salary, to $284,625.

Annual Performance-Based Bonus Opportunity

In addition to base salary, we provide the opportunity for each of our employees to earn annual performance-based cash bonuses. We provide this opportunity to encourage the achievement of corporate goals and to reward those employees who significantly impact our corporate results. The maximum annual bonus awards available to employees is represented by a percentage of each eligible employee s base salary. The maximum annual bonuses as a percentage of base salary, or the bonus percentage, for Messrs. Martino and Fuhrman is 50% and 30%, respectively, and the bonus percentage for Dr. Countouriotis is 30%.

Annual corporate goals are established by the board of directors taking into consideration the recommendations of the compensation committee. Our board of directors will generally consider each of our named executive officer s individual contributions towards reaching our annual corporate goals but does not typically establish specific individual goals for our named executive officers. The annual bonus payment, if any, that each of our named executive officers may earn each year is equal to annual base salary, multiplied by their bonus percentage and by the percent to which our corporate goals are achieved for such year.

In calculating the achievement of our corporate goals, the compensation committee and our board of directors reviews our performance against predetermined goal weightings assigned to each corporate goal. In order for a named executive officer to receive the maximum bonus, we must achieve 100% of our corporate goals.

The corporate goals established by the board of directors for 2012 and their relative weighting towards overall corporate goal achievement were: maximizing the value of our lead drug candidate, quizartinib (50%); building, retaining and nurturing an employee team (20%); implementing our financing strategy and managing expenses (20%); progress in at least one pipeline asset (5%); and implementing a business development strategy that involved concluding at least one partnership by the end of the year (5%).

No individual goals were established for any of our named executive officers for 2012. As a result, bonuses payable to each of our named executive officers for 2012 were based entirely on the achievement of corporate goals. For 2012, there was no minimum percentage of corporate goals that must be achieved in order to earn a bonus. In late 2012, the compensation committee and the board of directors considered each corporate goal in detail and determined that we had achieved 102% of the overall 2012 corporate goals due to:

- overachievement of our goal of maximizing the value of quizartinib, based on our achievement of gaining early access to our Phase 2 clinical trial data with agreement from our independent data monitoring committee, analyzing the Phase 2 trial and generating key tables and data to meet key operational milestones and gaining access to key historical databases owned and controlled by other entities; additionally, we continued to progress our regulatory strategy with regard to quizartinib;

- achievement of our goal of building, retaining and nurturing an employee team, based on development and roll-out of our vision, mission and core values, improvement in our employee engagement scores and through the addition of key clinical and regulatory personnel, including the addition of Dr. Countouriotis as our Chief Medical Officer;

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achievement of our goal of implementing a financing strategy, based on operating within budget, managing our cash reserves, closing the bridge financing in October and engaging and filing confidentially the first registration statement for our initial public offering prior to the end of 2012;

partial achievement of our goal to progress in at least one pipeline asset, as we developed two optimized lead compounds in our CSF1R program; and

no achievement of our business development goal, as we did not meet objectives related to progress in corporate collaborations and licensing activities.

Accordingly, in December 2012, we paid Messrs. Martino and Fuhrman and Dr. Countouriotis a bonus equal to the product of their base salary, multiplied by their bonus percentage and 102%. Dr. Countouriotis bonus was pro-rated for the period of time she provided services to us in 2012.

Long-Term Incentive Program

Prior to this offering, we have granted equity awards primarily through our 2011 amended and restated equity incentive plan, or 2011 pre-IPO plan. Our board of directors and stockholders adopted this plan to permit the grant of stock options and other stock awards to our officers, directors, employees and consultants. The material terms of our 2011 pre-IPO plan are further described under Employee Benefit Plans below.

All stock options were granted with an exercise price of no less than the fair market value of our common stock on the date of grant. In the absence of a public trading market for our common stock, our board of directors has determined the fair market value of our common stock in good faith based upon consideration of a number of relevant factors including the status of our development efforts, financial status, market conditions and with input from an independent third-party valuation specialist.

All option grants typically vest over four years, with one quarter of the shares subject to the stock option vesting on the one year anniversary of the vesting commencement date and the remaining shares vesting in equal months installments thereafter over three years. All options have a 10-year term. We may grant options that accelerate vesting in connection with certain change of control transactions and/or the option holder s termination of service. Additional information regarding accelerated vesting upon or following a change of control is discussed below under Termination-Based Compensation. Authority to make equity grants to our named executive officers rests with our board of directors or a committee thereof, which takes into account recommendation of our compensation committee, although our board of directors and compensation committee consider the recommendations of our chief executive officer for officers other than himself.

In August 2011, we commenced a one-time stock option repricing program, or the Repricing Program, whereby our employees (including our named executive officers), directors and consultants could elect to amend their out of the money stock options with an exercise price at or above $1,680.00 per share to reduce such exercise price to the greater of (i) $1,680.00 per share or (ii) the fair market value of our common stock as of the date of the repricing. The Repricing Program expired on September 30, 2011 and on this date an aggregate of 125 eligible options held by the named executive officers were amended to reduce the exercise price of such options to $1,680.00 per share. Except for the amendment to reduce the exercise price, each amended option remained subject to the same vesting schedule and other terms pursuant to which it was originally granted.

In connection with Mr. Martino and Dr. Countouriotis commencement of employment with us and in accordance with the terms of each of their respective employment or offer letter agreements, on January 12, 2012, the board of directors granted Mr. Martino an option to purchase 2,368 shares of our common stock and on April 24, 2012, the board of directors granted Dr. Countouriotis an option to purchase 520 shares of our common stock. In addition, on December 13, 2012, the board of directors granted to Messrs. Martino and Fuhrman and Dr. Countouriotis an option to purchase 451,899, 100,277 and 100,645 shares of our common stock, respectively.

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All of the stock options granted in 2012 vest over a four year period subject to the applicable named executive officer s continued service with us, as further described in the footnotes to the Outstanding Equity Awards at December 31, 2012 table below.

In connection with this offering, our board of directors has adopted new equity incentive plans described under Employee Benefit Plans below. The 2013 post-IPO plan will replace our existing 2011 pre-IPO plan immediately following this offering and, as described below, will provide us with greater flexibility in making a wide variety of equity awards.

Employment Agreements

We entered into employment agreements or offer letter agreements with each of Messrs. Martino and Fuhrman and Dr. Countouriotis in November 2011, September 2010 and January 2012, respectively. The agreements provide for at-will employment, base salary, incentive bonuses, standard employee benefit plan participation and recommendations for initial stock option grants. The agreements were each subject to execution of standard proprietary information and invention agreements and proof of identity and work eligibility in the United States.

Each of our named executive officers are entitled to certain severance and change of control benefits pursuant to their employment agreements or offer letters, as applicable, the terms of which are described below under Termination-Based Compensation.

Perquisites, Health, Welfare and Retirement Benefits

Our named executive officers are eligible to participate in all of our employee benefit plans, including our medical, dental, vision, group life and disability insurance plans, in each case on the same basis as our other employees. Additionally, we pay the premiums for term life insurance for all of our employees, including our named executive officers, and we pay the premiums for supplemental long term disability exclusively for our executive officers, including our named executive officers.

None of our named executive officers participate in or have account balances in qualified or non-qualified defined benefit, non-qualified defined contribution plans or pension plans sponsored by us. We provide a 401(k) plan for our eligible employees, including our named executive officers, as described in the section below entitled 401(k) Plan .

Termination-Based Compensation

Regardless of the manner in which a named executive officer s employment terminates, the named executive officer is entitled to receive amounts earned during his term of employment, including salary and unused vacation pay. In addition, each of our named executive officers is or was entitled to the severance and change of control benefits described below.

In November 2011, we entered into an employment agreement with Mr. Martino, our President and Chief Executive Officer, which was amended in January 2012. Mr. Martino s employment agreement provides that if we terminate Mr. Martino without cause not upon or within twelve months following a change of control transaction, he is entitled to: (i) severance payments at a rate equal to his base salary then in effect for a period of six months following his termination date, as well as a pro rata portion of the performance bonus he would have been entitled to for the year of termination (provided he was employed for at least six months of such calendar year), (ii) payment of COBRA health insurance premiums for the lesser of (x) a period of one year following his termination date or (y) the period until he commences full-time employment with another company and becomes eligible to participate in such company s health insurance plan, and (iii) accelerated vesting of 100% of the shares subject to the stock option to purchase 2,368 shares awarded to Mr. Martino in connection with his commencement of employment that was granted in January 2012. However, if his employment with us or our

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successor is terminated upon or within twelve months following a change of control transaction by us without cause, Mr. Martino will receive the severance benefits described above, except the severance payments described in (i) above will be paid for a severance period of 12 months rather than six months.

In addition, pursuant to an agreement entered into in November 2011, Mr. Martino is entitled to receive a success fee upon a change of control transaction that occurs while he is serving as our chief executive officer or during the six month period following his termination without cause, in an amount based on the value of his vested equity ownership, as a percentage of our fully-diluted capital, and the consideration paid to our stockholders in a change of control transaction. The success fee is payable in the same forms of consideration and in the same proportions as paid to us and our stockholders in connection wit the change of control transaction. This success fee arrangement will terminate in connection with the effectiveness of this offering.

In September 2010, we entered into an employment offer letter with Mr. Fuhrman, our Chief Financial Officer, which was amended in December 2012. Mr. Fuhrman s employment agreement provides if either (a) we terminate Mr. Fuhrman without cause or (b) Mr. Fuhrman s employment with us or our successor is terminated by him or by us following a change of control transaction because he was not offered a position in the greater San Diego, California metropolitan area involving status, duties, salary and benefits substantially equivalent to those enjoyed by Mr. Fuhrman in his then-existing position with us, he will be entitled to: (i) severance payments at a rate equal to his base salary then in effect for a period of one-year following his termination date, (ii) payment of COBRA health insurance premiums for the lesser of (x) a period of one year following his termination date or (y) the period until he commences full-time employment with another company and becomes eligible to participate in such company s health insurance plan, and (iii) acceleration of vesting of any options or restricted stock then held by Mr. Fuhrman, with respect to a termination described in (a) above, to the extent that such options and restricted stock would have vested during the one year period following termination, and with respect to a termination described in (b) above, in full. In addition, pursuant to an agreement entered into in January 2012, Mr. Fuhrman is entitled to receive a success fee upon a change of control transaction based on his vested equity ownership on terms substantially similar to the success fee arrangement described above for Mr. Martino. As with Mr. Martino, this success fee arrangement will terminate in connection with the effectiveness of this offering.

In January 2012, we entered into an employment offer letter with Dr. Countouriotis, our Chief Medical Officer, which provides that if we terminate Dr. Countouriotis without cause, she will be entitled to: (i) severance payments at a rate equal to her base salary then in effect for a period of six months following her termination date and (ii) payment of COBRA health insurance premiums for the lesser of (x) a period of one year following her termination date or (y) the period until she commences full-time employment with another company and becomes eligible to participate in such company s health insurance plan. In addition, if during her employment with us or our successor is terminated by her or by us within the twelve months following a change of control transaction, 100% of the shares subject to option awards granted under our 2011 pre-IPO plan and then-held by to Dr. Countouriotis will fully vest. In addition, Dr. Countouriotis will be entitled to payment of up to $60,000 if before February 2014 she is terminated without cause following a change of control transaction prior to February 2014. This special payment will expire upon the effectiveness of this offering.

In addition, pursuant to an agreement entered into in January 2012, Dr. Countouriotis is entitled to receive a success fee upon a change of control transaction based on her vested equity ownership on terms substantially similar to the success fee arrangement described above for Mr. Martino. As with Mr. Martino, this success fee arrangement will terminate in connection with the effectiveness of this offering.

For purposes of Mr. Martino s employment agreement and success fee arrangement, Mr. Fuhrman s employment offer letter, and Dr. Countouriotis employment offer letter and success fee arrangement:

cause generally means the occurrence of any of the following with respect to each executive: (i) conviction of any felony or any crime involving fraud or dishonesty that has a material adverse

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effect on us, (ii) participation (whether by affirmative act or omission) in a fraud, act of dishonesty or other act of misconduct against us and/or a parent or subsidiary of us, (iii) conduct which, based upon a good faith and reasonable factual investigation by us demonstrates gross unfitness to serve, (iv) violation of any statutory or fiduciary duty, or duty of loyalty, owed to us and/or a parent or subsidiary of us, (v) breach of any material term of any material contract with us and/or a parent or subsidiary of us, and (vi) repeated violation of any material company policy.

change of control transaction for purposes Mr. Martino s employment agreement and success fee agreement and Mr. Fuhrman s and Dr. Countouriotis employment offer letters generally means a (i) a merger or consolidation following which our stockholders do not own at least a majority of the surviving corporation s voting shares; (ii) a sale of all or substantially all of our assets or (iii) a sale or other transfer of our capital stock as a result of which our stockholders cease to own at least a majority of the surviving corporation s voting shares, other than a transaction involving the issuance of our shares primarily for the purpose of raising capital for us.

We have routinely granted and will continue to grant our named executive officers stock options under our equity incentive plans. For a description of the change of control provisions in such equity incentive plans applicable to these stock options, see Employee Benefit Plans 2011 Amended and Restated Equity Incentive Plan (2011 pre-IPO plan) and 2013 Equity Incentive Plan (2013 post-IPO plan) below.

Outstanding Equity Awards at December 31, 2012

The following table sets forth certain information regarding outstanding equity awards granted to our named executive officers that remained outstanding as of December 31, 2012.

Name	Grant Date	Number of securities underlying unexercised options (#) Exercisable	Number of securities underlying unexercised options (#) Unexercisable	Option Exercise Price ($)[1]	Option Expiration Date
Michael A. Martino	1/12/2012	642	1,726[2]	600.00	1/11/2022
	12/13/2012	9,415	442,484[3]	6.00	12/12/2022
Alan Fuhrman	10/11/2010	68	57[4]	1,680.00[5]	10/10/2020
	8/18/2011	126	188[6]	600.00	8/17/2021
	12/13/2012	2,089	98,188[3]	6.00	12/12/2022
Athena Countouriotis, M.D.	4/24/2012		520[7]	600.00	4/23/2022
	12/13/2012	2,097	98,548[3]	6.00	12/12/2022

(1) At the time of grant, all of the stock options had a per share exercise price equal to the fair market value of one share of our common stock on the date of grant, as determined in good faith by our board of directors with the assistance of a third-party valuation expert. Vesting is subject to the applicable named executive officer s continued services with us through each of the vesting dates.

(2) The option vests at the rate of 25% of the total number of shares subject to the option on the one year anniversary of November 9, 2011 and 1/48th of the total number of shares subject to the option each month thereafter, subject to acceleration in connection with certain termination and change of control transactions pursuant to Mr. Martino s employment agreement, as described in the section above entitled Termination Based Compensation .

(3) The option vests at the rate of 1/48th of the total number of shares subject to the option each month, commencing on November 1, 2012. The option granted to Dr. Countouriotis is subject to acceleration in connection with certain termination and change of control transactions pursuant to Dr. Countouriotis employment agreement, as described in the section above entitled Termination Based Compensation .

(4) The option vests at the rate of 25% of the total number of shares subject to the option on the one year anniversary of the grant date and 1/48th of the total number of shares subject to the option each month thereafter and is subject to acceleration in connection with certain termination and change of control

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transactions pursuant to Mr. Fuhrman s offer letter, as described in the section above entitle Termination Based Compensation .

(5) The option was granted with an exercise price equal to $3,696.00 per share, which was reduced to $1,680.00 per share in connection with the Repricing Program, as described in the section below entitled Long-Term Incentive Program .

(6) The option vests at the rate of 25% of the total number of shares subject to the option on the one year anniversary of May 18, 2011 and 1/48th of the total number of shares subject to the option each month thereafter. In addition, the option will become vested and exercisable in full upon Mr. Fuhrman s termination without cause or constructive termination within twelve months following a change of control transaction.

(7) The option vests at the rate of 25% of the total number of shares subject to the option on the one year anniversary of February 7, 2012 and 1/48th of the total number of shares subject to the option each month thereafter, subject to acceleration in connection with certain termination and change of control transactions pursuant to Dr. Countouriotis employment agreement, as described in the section above entitled Termination-Based Compensation .

Option Exercises and Stock Vested

Our named executive officers did not exercise any stock option awards during the year ended December 31, 2012 or prior to the date of this prospectus.

2011 Amended and Restated Equity Incentive Plan

Our board of directors and our stockholders approved our 2011 amended and restated equity incentive plan, or the 2011 pre-IPO plan, which became effective in January 2011, as an amendment and restatement of our 2001 Equity Incentive Plan, which was originally approved by our board of directors and stockholders in 2001. The 2011 pre-IPO plan will terminate in January 2021, unless our board of directors terminates it earlier. After the effective date of the 2013 post-IPO plan described below, no additional awards will be granted under the 2011 pre-IPO plan, and all awards granted under the 2011 pre-IPO plan that are repurchased, forfeited, expire or are cancelled will become available for grant under the 2013 post-IPO plan in accordance with its terms.

The principal purpose of the 2011 pre-IPO plan is to attract, retain and motivate selected employees, consultants and directors of us and our affiliates through the granting of the following:

Incentive stock options within the meaning of Section 422 of the Code, or ISOs, which may be granted solely to our employees, including officers; and

Nonstatutory stock options, or NSOs, stock bonus awards, and restricted stock awards, which may be granted to our directors, consultants or employees, including officers.

The principal features of the 2011 pre-IPO plan are summarized below. This summary is qualified in its entirety by reference to the text of the 2011 pre-IPO plan, which is filed as an exhibit to the registration statement of which this prospectus is a part.

Share Reserve. The 2011 pre-IPO plan currently reserves up to 1,220,480 shares of common stock for issuance which consists of 1,214,212 shares underlying options outstanding as of March 31, 2013 and 151 shares underlying options that have been exercised. Additionally, the 2011 pre-IPO plan provides that no more than 2,440,960 shares may be issued under the plan pursuant to the exercise of ISOs. Shares of our common stock subject to options and other stock awards that have expired or otherwise terminate under the 2011 pre-IPO plan without having been exercised in full will become available for grant under the 2011 pre-IPO plan. Shares of our common stock issued under the 2011 pre-IPO plan may include previously unissued shares or reacquired shares bought on the market or otherwise.

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As of March 31, 2013, an aggregate of 6,117 shares of our common stock remained available for future grant under our 2011 pre-IPO plan and options granted pursuant to our 2011 pre-IPO plan to purchase an aggregate of 1,214,212 were outstanding.

Administration. The 2011 pre-IPO plan is administered by our board of directors, which may in turn delegate authority to administer the plan to a committee of one or more members of the board. Subject to the terms of the 2011 pre-IPO plan, our board of directors or its authorized committee, referred to herein as the plan administrator, determines recipients, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting. Subject to the limitations set forth below, our plan administrator will also determine the exercise price of options granted under the 2011 pre-IPO plan.

Stock Options. Stock options will be granted pursuant to stock option agreements. Generally, the exercise price for an ISO or NSO will not be less than 100% of the fair market value of the common stock subject to the option on the date of grant. Options granted under the 2011 pre-IPO plan will vest at the rate specified in the option agreement. A stock option agreement may provide for early exercise, prior to vesting, rights of repurchase, and rights of first refusal. Unvested shares of our common stock issued in connection with an early exercise may be repurchased by us.

In general, the term of stock options granted under the 2011 pre-IPO plan may not exceed 10 years. Unless the terms of an optionholder s stock option agreement provide for earlier or later termination, if an optionholder s service relationship with us, or any affiliate of ours, ceases due to disability or death, the optionholder, or his or her beneficiary, may exercise any vested options up for to 12 months, or 18 months in the event of death, after the date the service relationship ends, unless the terms of the stock option agreement provide for earlier termination. If an optionholder s service relationship with us, or any affiliate of ours, ceases without cause for any reason other than disability or death, the optionholder may exercise any vested options for up to three months after the date the service relationship ends, unless the terms of the stock option agreement provide for a longer or shorter period to exercise the option. If an optionholder s service relationship with us, or any affiliate of ours, ceases with cause, the option will terminate at the time the optionholder s relationship with us ceases. In no event may an option be exercised after its expiration date.

Acceptable forms of consideration for the purchase of our common stock under the 2011 pre-IPO plan include: (i) cash and (ii) at the discretion of our plan administrator at the time of grant, common stock previously owned by the optionholder, deferred payment arrangements, or other legal consideration approved by our plan administrator.

Limitations. The aggregate fair market value, determined at the time of grant, of shares of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. The options or portions of options that exceed this limit are treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any affiliate unless the following conditions are satisfied:

- the option exercise price must be at least 110% of the fair market value of the stock subject to the option on the date of grant; and
- the term of any ISO award must not exceed five years from the date of grant.

Corporate Transactions. Unless otherwise provided in the stock award agreement, in the event of certain corporate transactions, any or all outstanding stock awards under the 2011 pre-IPO plan may be assumed, continued or substituted for by any surviving entity. If the surviving entity elects not to assume, continue or substitute for such awards, the vesting provisions of such stock awards generally will be accelerated in full and such stock awards will be terminated if and to the extent not exercised at or prior to the effective time of the

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corporate transaction and our repurchase rights will generally lapse. In the event of our dissolution or liquidation, all outstanding stock awards under the 2011 pre-IPO plan will terminate immediately prior to such event.

Under the 2011 pre-IPO plan, a corporate transaction generally means the consummation of (i) a sale of substantially all of our assets; (ii) a merger or consolidation in which we are not the surviving corporation; (iii) a merger in which we are the surviving corporation but the shares or our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction; or (iv) any transaction that results in the transfer of more than 50% of our voting power.

Plan Amendments. Our plan administrator has the authority to amend, suspend or terminate the 2011 plan. However, no amendment or termination of the plan may adversely affect any rights under awards already granted to a participant unless agreed to by the affected participant. We will obtain stockholder approval of any amendment to the 2011 pre-IPO plan as required by applicable law.

2013 Equity Incentive Plan

Our board of directors adopted the 2013 equity incentive plan, or the 2013 post-IPO plan in February 2013, and we expect our stockholders will approve the plan prior to this offering and that the 2013 post-IPO plan will become effective upon the execution and delivery of the underwriting agreement for this offering. Once the 2013 post-IPO plan is effective, no further grants will be made under the 2011 pre-IPO plan.

Stock Awards. The 2013 post-IPO plan provides for the grant of ISO, NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based stock awards, and other forms of equity compensation, or collectively, stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. Additionally, the 2013 post-IPO plan provides for the grant of performance cash awards. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

Share Reserve. Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under the 2013 post-IPO plan after the 2013 post-IPO plan becomes effective is the sum of (i) 625,000 shares, plus (ii) the number of shares reserved for issuance under our 2011 pre-IPO plan at the time our 2013 post-IPO plan becomes effective, plus (iii) any shares subject to stock options or other stock awards granted under our 2011 pre-IPO plan that would have otherwise returned to our 2011 pre-IPO plan (such as upon the expiration or termination of a stock award prior to vesting). Additionally, the number of shares of our common stock reserved for issuance under our 2013 post-IPO plan will automatically increase on January 1 of each year, beginning on January 1, 2014 (assuming the 2013 post-IPO plan becomes effective before such date) and continuing through and including January 1, 2023, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. The maximum number of shares that may be issued upon the exercise of ISOs under our 2013 post-IPO plan is 1,250,000 shares.

No person may be granted stock awards covering more than 875,000 shares of our common stock under our 2013 post-IPO plan during any calendar year pursuant to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value on the date the stock award is granted. Additionally, no person may be granted in a calendar year a performance stock award covering more than 875,000 shares or a performance cash award having a maximum value in excess of $1,000,000. Such limitations are designed to help assure that any deductions to which we would otherwise be entitled with respect to such awards will not be subject to the $1,000,000 limitation on the income tax deductibility of compensation paid to any covered executive officer imposed by Section 162(m) of the Code.

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If a stock award granted under the 2013 post-IPO plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2013 post-IPO plan. In addition, the following types of shares under the 2013 post-IPO plan may become available for the grant of new stock awards under the 2013 post-IPO plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under the 2013 post-IPO plan may be previously unissued shares or reacquired shares bought by us on the open market. As of the date hereof, no awards have been granted and no shares of our common stock have been issued under the 2013 post-IPO plan.

Administration. Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2013 post-IPO plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2013 post-IPO plan, our board of directors or the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2013 post-IPO plan. Subject to the terms of our 2013 post-IPO plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Stock Options. Incentive and nonstatutory stock options are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2013 post-IPO plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2013 post-IPO plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2013 post-IPO plan, up to a maximum of 10 years. Unless the terms of an option holder s stock option agreement provide otherwise, if an option holder s service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the option holder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder s service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder s death.

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Tax Limitations on Incentive Stock Options. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the term of the ISO does not exceed five years from the date of grant.

Restricted Stock Awards. Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates, or (3) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the plan administrator. Rights to acquire shares under a restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock unit awards that have not vested will be forfeited upon the participant s cessation of continuous service for any reason.

Restricted Stock Unit Awards. Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant s cessation of continuous service for any reason.

Stock Appreciation Rights. Stock appreciation rights are granted pursuant to stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a stock appreciation unit, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a stock appreciation unit, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the stock appreciation unit is exercised. A stock appreciation unit granted under the 2013 post-IPO plan vests at the rate specified in the stock appreciation grant agreement as determined by the plan administrator.

The plan administrator determines the term of stock appreciation rights granted under the 2013 post-IPO plan, up to a maximum of ten years. Unless the terms of a participant s stock appreciation right agreement provides otherwise, if a participant s service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant s service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested stock appreciation right for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

Performance Awards. The 2013 post-IPO plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the $1,000,000 limitation on the income tax deductibility of compensation paid to a covered executive officer imposed by Section 162(m) of the Code. To help assure that the compensation attributable to performance-based awards will so qualify, our compensation

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committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected will be based on one or more of the following criteria: (1) earnings (including earnings per share and net earnings); (2) earnings before interest, taxes and depreciation; (3) earnings before interest, taxes, depreciation and amortization; (4) earnings before interest, taxes, depreciation, amortization and legal settlements; (5) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (6) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (7) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (8) total stockholder return; (9) return on equity or average stockholder s equity; (10) return on assets, investment, or capital employed; (11) stock price; (12) margin (including gross margin); (13) income (before or after taxes); (14) operating income; (15) operating income after taxes; (16) pre-tax profit; (17) operating cash flow; (18) sales or revenue targets; (19) increases in revenue or product revenue; (20) expenses and cost reduction goals; (21) improvement in or attainment of working capital levels; (22) economic value added (or an equivalent metric); (23) market share; (24) cash flow; (25) cash flow per share; (26) share price performance; (27) debt reduction; (28) implementation or completion of projects or processes; (29) user satisfaction; (30) stockholders equity; (31) capital expenditures; (32) debt levels; (33) operating profit or net operating profit; (34) workforce diversity; (35) growth of net income or operating income; (36) billings; (37) bookings; (38) the number of users, including but not limited to unique users; (39) employee retention; (40) and to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our board of directors.

The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (i) in the award agreement at the time the award is granted or (ii) in such other document setting forth the performance goals at the time the goals are established, we will appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any extraordinary items as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger , consolidation, spin-off, combination or exchange of shares or other similar corporate change, or distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; and (12) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

Other Stock Awards. The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

Changes to Capital Structure. In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (a) the class and maximum number of shares reserved for issuance under the 2013 post-IPO plan, (b) the class and maximum number of shares by which the share reserve may increase automatically each year, (c) the class and maximum number of

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shares that may be issued upon the exercise of ISOs, (d) the class and maximum number of shares subject to stock awards that can be granted in a calendar year (as established under the 2013 post-IPO plan pursuant to Section 162(m) of the Code) and (e) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

Corporate Transactions. In the event of certain specified significant corporate transactions, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or parent company;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring entity or parent company;
- accelerate the vesting of the stock award and provide for its termination prior to the effective time of the corporate transaction;
- arrange for the lapse of any reacquisition or repurchase right held by us;
- cancel or arrange for the cancellation of the stock award in exchange for such cash consideration, if any, as our board of directors may deem appropriate; or
- make a payment equal to the excess of (a) the value of the property the participant would have received upon exercise of the stock award over (b) the exercise price otherwise payable in connection with the stock award.

Our plan administrator is not obligated to treat all stock awards, even those that are of the same type, in the same manner.

Under the 2013 post-IPO plan, a corporate transaction is generally the consummation of (i) a sale or other disposition of all or substantially all of our consolidated assets, (ii) a sale or other disposition of at least 90% of our outstanding securities, (iii) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (iv) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change of Control. The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2013 post-IPO plan, a change of control is generally (i) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (ii) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity; (iii) a consummated sale, lease or exclusive license or other disposition of all or substantially of our consolidated assets; or (iv) when a majority of the board of directors becomes comprised of individuals whose nomination, appointment, or election was not approved by a majority of the board members or their approved successors.

Amendment and Termination. Our board of directors has the authority to amend, suspend, or terminate our 2013 post-IPO plan, provided that such action does not materially impair the existing rights of any participant without such participant s written consent. No ISOs may be granted after the tenth anniversary of the date our board of directors adopted our 2013 post-IPO plan.

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2013 Employee Stock Purchase Plan

Our board of directors adopted our 2013 Employee Stock Purchase Plan, or the ESPP, in February 2013, and we expect our stockholders will approve the ESPP prior to the closing of this offering. The ESPP will become effective immediately upon the signing of the underwriting agreement related to this offering. The purpose of the ESPP is to retain the services of new employees and secure the services of new and existing employees while providing incentives for such individuals to exert maximum efforts toward our success and that of our affiliates.

Share Reserve. Following this offering, the ESPP authorizes the issuance of 125,000 shares of our common stock pursuant to purchase rights granted to our employees or to employees of any of our designated affiliates. The number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, from January 1, 2014 (assuming the ESPP becomes effective before such date) through January 1, 2023, by the least of (a) 1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, (b) 166,666 shares, or (c) a number determined by our board of directors that is less than (a) or (b). The ESPP is intended to qualify as an employee stock purchase plan within the meaning of Section 423 of the Code. As of the date hereof, no shares of our common stock have been purchased under the ESPP.

Administration. Our board of directors has delegated its authority to administer the ESPP to our compensation committee. The ESPP is implemented through a series of offerings of purchase rights to eligible employees. Under the ESPP, we may specify offerings with duration of not more than 27 months, and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering may be terminated under certain circumstances.

Payroll Deductions. Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings for the purchase of our common stock under the ESPP. Unless otherwise determined by our board of directors, common stock will be purchased for accounts of employees participating in the ESPP at a price per share equal to the lower of (a) 85% of the fair market value of a share of our common stock on the first date of an offering or (b) 85% of the fair market value of a share of our common stock on the date of purchase.

Limitations. Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our board of directors: (a) customarily employed for more than 20 hours per week, (b) customarily employed for more than five months per calendar year or (c) continuous employment with us or one of our affiliates for a period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of $25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value pursuant to Section 424(d) of the Code.

Changes to Capital Structure. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or similar transaction, the board of directors will make appropriate adjustments to (a) the number of shares reserved under the ESPP, (b) the maximum number of shares by which the share reserve may increase automatically each year and (c) the number of shares and purchase price of all outstanding purchase rights.

Corporate Transactions. In the event of certain significant corporate transactions, including: (i) a sale of all our assets, (ii) the sale or disposition of 90% of our outstanding securities, (iii) the consummation of a merger or

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consolidation where we do not survive the transaction, and (iv) the consummation of a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue or substitute for such purchase rights, then the participants accumulated payroll contributions will be used to purchase shares of our common stock within 10 business days prior to such corporate transaction, and such purchase rights will terminate immediately. A corporate transaction generally has the same meaning as such term in the 2013 post-IPO plan.

Plan Amendments, Termination. Our board of directors has the authority to amend or terminate our ESPP, provided that except in certain circumstances any such amendment or termination may not materially impair any outstanding purchase rights without the holder s consent. We will obtain stockholder approval of any amendment to our ESPP as required by applicable law or listing requirements.

401(k) Plan

We maintain a defined contribution employee retirement plan for our employees. The plan is intended to qualify as a tax-qualified plan under Section 401(a) of the Code, or the 401(k) plan, so that contributions to the 401(k) plan, and income earned on such contributions, are not taxable to participants until withdrawn or distributed from the 401(k) plan. The 401(k) plan provides that each participant may contribute up to 100% of his or her pre-tax eligible compensation, up to a statutory limit, which is $17,000 for 2012. Participants who are at least 50 years old can also make catch-up contributions, which in 2012 may be up to an additional $5,500 above the statutory limit. Under the 401(k) plan, each employee is fully vested in his or her deferred salary contributions. Employee contributions are held and invested by the plan s trustee. The 401(k) plan also permits us to make discretionary contributions and matching contributions, subject to established limits and a vesting schedule. To date, we have not made any discretionary or matching contributions to the plan on behalf of participating employees. The 401(k) plan currently does not offer the ability to invest in our securities.

Non-Employee Director Compensation

We compensate Mr. Hasnain and we previously compensated Mr. Zarrabian for their services as non-employee members of our board of directors. We provided cash fees pursuant to agreements with each of Mr. Hasnain and Mr. Zarrabian, which are described below under Compensation Agreements with Directors .

In addition, our board of directors approved an option grant on December 13, 2012 under our 2011 pre-IPO plan to Mr. Zarrabian covering 10,064 shares of our common stock and to Mr. Hasnain covering 144,259 shares of our common stock. Each of these options were granted with an exercise price equal to $6.00 per share, which the board of directors determined was the fair market value of our common stock on such date. Mr. Zarrabian s option grant was fully vested on the date of grant and Mr. Hasnain s option grant vests monthly over a four year period, subject to Mr. Hasnain s continuous service with us through such dates, provided that, if during such time there is a covered transaction, the option will vest in full. A covered transaction for purposes of this stock option grant and for purposes of Mr. Hasnain s and Mr. Zarrabian s agreement described below under Non-Employee Director Compensation Plans and Agreements generally means (i) our merger or consolidation following which our stockholders cease to own at least a majority of the surviving corporation s voting shares; (ii) a sale of all or substantially all of our assets or (iii) a sale or other transfer of shares of our capital stock resulting in our stockholder ceasing to own at least a majority of the surviving corporation s voting shares, other than a transaction involving the issuance of our shares primarily for the purpose of raising capital.

Directors who are also our employees generally do not receive cash or equity compensation for service on our board of directors in addition to the compensation payable for their service as our employees. Our non-employee directors who are affiliated with our principal investors do not receive cash or equity compensation for service on our board of directors. However, we have reimbursed and will continue to reimburse

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all of our non-employee directors for their travel, lodging and other reasonable expenses incurred in attending meetings of our board of directors and committees of the board of directors.

The following table sets forth in summary form information concerning the compensation that we paid or awarded during the year ended December 31, 2012 to each of our non-employee directors: Dr. Parkinson was not a member of the board of directors in 2012 and is not included in the following table.

Name[1]	Fees Earned or Paid in Cash ($)	Option Awards ($)[2]	Total ($)
David P. Bonita, M.D.[3]			
Steven A. Elms			
Standish M. Fleming			
Faheem Hasnain	118,000	522,102[4]	640,102
Allan P. Marchington, Ph.D.			
Isai Peimer			
Joseph Regan			
Saiid Zarrabian[5]	46,781	11,425[6]	58,206

(1) Mr. Martino was an employee director during 2012 and his compensation is fully reflected in the Summary Compensation Table above.

(2) Amounts listed represent the aggregate fair value amount computed as of the grant date of the option awards granted during 2012 in accordance with FASB ASC Topic 718. Assumptions used in the calculation of these amounts are included in Note 7, Stock Based Compensation, of the Notes to our Consolidated Financial Statements. As required by SEC rules, the amounts shown exclude the impact of estimated forfeitures related to service-based vesting conditions. Our directors will only realize compensation to the extent the trading price of our common stock is greater than the exercise price of such stock options. Other than Messrs. Hasnain and Zarrabian, none of our non-employee directors held stock option awards as of December 31, 2012.

(3) Dr. Bonita joined our board in October 2012.

(4) Represents an option to purchase 144,259 shares granted to Mr. Hasnain during 2012 for service on our board of directors. The shares subject to this option vest at a rate of 1/48 each month following November 1, 2012, subject to Mr. Hasnain s continued service with us, provided that, if during such time there is a covered transaction, the option will vest in full. As of December 31, 2012, an aggregate of 145,096 shares were outstanding under all options to purchase our common stock held by Mr. Hasnain.

(5) Mr. Zarrabian resigned as a member of our board on December 19, 2012.

(6) Represents and option to purchase 10,064 shares granted to Mr. Zarrabian during 2012 for service on our board of directors. The shares subject to this option were fully vested on the date of grant and will remain exercisable for a period of one year following Mr. Zarrabian s resignation as a member of our board. As of December 31, 2012, an aggregate of 10,208 shares were outstanding under all options to purchase our common stock held by Mr. Zarrabian.

Non-Employee Director Compensation Plans and Agreements

In 2010, we entered into a board compensation letter agreement with Faheem Hasnain in connection with his appointment as one of our directors, which we subsequently amended in April 2011 and January 2012. Pursuant to the letter agreement, Mr. Hasnain receives a fee of $50,000 per year as compensation for his services as Chairman of our board of directors, $2,500 for each regularly scheduled meeting of the board with a scheduled duration of less than a full day that he attends in person, $500 or each meeting of the board that he attends by telephone, and $3,000 for each meeting of the board with a scheduled duration of a full day session (or longer) that he attends in person. Mr. Hasnain was granted an option to purchase up to 62 shares of our common stock under our 2011 pre-IPO plan in 2010, which vested over a four year period for so long as Mr. Hasnain continues to serve on the board of directors, provided that, in the event of a covered transaction during the period of Mr. Hasnain s service he is entitled to full acceleration of all unvested stock options then held by him. The

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amended board compensation letter agreement provided for an additional retention option grant to Mr. Hasnain under the 2011 pre-IPO plan to purchase up to 775 shares of our common stock that vests monthly over a three year period for so long as Mr. Hasnain continues to serve on the board, provided that, in the event of a covered transaction during the period of Mr. Hasnain s service he would be entitled to full acceleration of all unvested stock options then held by him. Following the closing of this offering, we intend to amend this agreement with Mr. Hasnain to align the cash compensation with our Non-Employee Director Compensation Policy described below.

In June 2009, we entered into a letter agreement with Mr. Zarrabian in connection with his appointment as one of our directors. Pursuant to the letter agreement, Mr. Zarrabian received a fee of $17,500 per year as compensation for his services as a non-employee member of our board of directors, $2,500 for each regularly scheduled meeting of the board that he attended in person, $500 for each regularly scheduled meeting of the board that he attended by telephone, and up to $3,000 per day for attendance in person or by telephone at any special meeting of the board, for time spent serving on committees of the board or for time spent performing other services as a non-employee member of the board. The letter agreement also provided for the grant to Mr. Zarrabian of an option to purchase up to 41 shares of our common stock under the terms of our 2011 pre-IPO plan. The shares subject to the option vested in a series of 36 successive equal monthly installments during Mr. Zarrabian s service on the board provided that, in the event of a covered transaction during the period of Mr. Zarrabian s service he was entitled to full acceleration of all unvested stock options then held by him. Mr. Zarrabian resigned from our board of directors in December 2012.

In addition, Mr. Hasnain and Mr. Zarrabian are each entitled to receive a success fee upon a covered transaction that occurs while Mr. Hasnain is serving as the Chairman of our board or directors or while Mr. Zarrabian was serving as a member of our board of directors, as applicable, (or within six months after termination of such service), in an amount based on the value of each of Mr. Hasnain s and Mr. Zarrabian s vested equity ownership, as a percentage of our fully-diluted capital, and the consideration paid to our stockholders in a change of control transaction. The success fee is payable in the same forms of consideration and in the same proportions as paid to us and our stockholders in connection with the change of control transaction. This success fee arrangement will terminate in connection with the effectiveness of this offering.

In February 2013, our board of directors adopted a compensation program for our non-employee directors, or the Non-Employee Director Compensation Policy. The Non-Employee Director Compensation Policy will be effective on the effective date of the underwriting agreement for this offering. The Non-Employee Director Compensation Policy will apply to each of our non-employee directors who our compensation committee determines is eligible to receive compensation under the policy and may be amended by the compensation committee at any time. Pursuant to the Non-Employee Director Compensation Policy, each eligible non-employee member of our board of directors will receive the following cash compensation for board services, as applicable:

- $40,000 per year for service as a board member;
- an additional $30,000 per year for service as the chairman of the board; and
- an additional $10,000 per year for service as the chairman of each of the audit committee and compensation committee.

In addition, our eligible non-employee directors will receive initial and annual, automatic, non-discretionary grants of nonqualified stock options under the terms and provisions of our 2013 post-IPO plan.

Each eligible non-employee director currently serving on our board on the closing of this offering or joining our board after the closing of this offering will automatically be granted a non-statutory stock option to purchase 7,085 shares of common stock with an exercise price equal to the then fair market value of our common stock under our 2013 post-IPO plan. Each of these initial grants will vest in equal annual installments over a three year period, subject to the director s continued service with us through such dates. On the date of each annual meeting of our stockholders beginning in 2014, each non-employee director will automatically be granted

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a non-statutory stock option to purchase 3,750 shares of common stock on that date with an exercise price equal to the then fair market value of our common stock under our 2013 post-IPO plan. The annual grants will vest in full on the one-year anniversary of the date of grant, subject to the director s continued service with us through such date. All stock options granted will have a maximum term of 10 years and will vest in full upon the closing of a change of control transaction.

For a more detailed description of our 2013 post-IPO plan, see Employee Benefit Plans above.

Limitation of Liability and Indemnification

Our amended and restated certificate of incorporation, which will become effective upon the closing of this offering, limits the liability of directors to the maximum extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for breach of their fiduciary duties as directors, except for liability for any:

- breach of their duty of loyalty to the corporation or its stockholders;
- act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- transaction from which the directors derived an improper personal benefit.

Our amended and restated certificate of incorporation does not eliminate a director s duty of care and, in appropriate circumstances, equitable remedies, such as injunctive or other forms of non-monetary relief, remain available under Delaware law. These limitations also do not affect a director s responsibilities under any other laws, such as the federal securities laws or other state or federal laws. Our amended and restated bylaws, which will become effective upon the closing of this offering, provide that we will indemnify our directors and executive officers, and may indemnify other officers, employees and other agents, to the fullest extent permitted by law. Our amended and restated bylaws also provide that we are obligated to advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding and also permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in connection with their services to us, regardless of whether our amended and restated bylaws permit such indemnification. We have obtained a directors and officers liability insurance policy.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors and executive officers, in addition to the indemnification provided for in our amended and restated bylaws. These agreements, among other things, require us to indemnify our directors and executive officers for certain expenses, including attorneys fees, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of their services as one of our directors or executive officers, or any of our subsidiaries or any other company or enterprise to which the person provides services at our request. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. A stockholder s investment may be harmed to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

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CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2010 to which we have been a party, in which the amount involved exceeded or will exceed $120,000, and in which any of our directors, executive officers or, beneficial owners of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest, other than equity or other compensation, termination, change-in-control arrangements and other arrangements, which are described under Executive and Director Compensation. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, in arm s-length transactions.

Policies and Procedures for Transactions with Related Persons

We are in the process of adopting a written Related-Person Transactions Policy that sets forth our policies and procedures regarding the identification, review, consideration, approval and oversight of related-person transactions. For purposes of our policy only, a related-person transaction is a past, present or future transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we and any related person are participants, the amount involved exceeds $120,000 and a related person has a direct or indirect material interest. Transactions involving compensation for services provided to us as an employee, director, consultant or similar capacity by a related person are not covered by this policy. A related person, as determined since the beginning of our last fiscal year, is any executive officer, director or nominee to become director, a holder of more than 5% of our common stock, including any immediate family members of such persons or any entity in which such a person has a 10% or greater equity interest. Any related-person transaction may only be consummated if our audit committee has approved or ratified the transaction in accordance with the policy guidelines set forth below.

The policy imposes an affirmative duty upon each director and executive officer to identify, and we will request that significant stockholders identify, any transaction involving them, their affiliates or immediate family members that may be considered a related party transaction before such person engages in the transaction. Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our audit committee (or, where review by our audit committee would be inappropriate, to another independent body of our board of directors) for review. The presentation must include a description of, among other things, the material facts, the direct and indirect interests of the related persons, the benefits of the transaction to us and whether any alternative transactions are available. In considering related-person transactions, our audit committee takes into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;
- the impact on a director s independence in the event the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the terms of the transaction;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from our employees generally.

In the event a director has an interest in the proposed transaction, the director must recuse himself or herself from the deliberations and approval process. Our policy requires that, in reviewing a related-person transaction, our audit committee must consider, in light of known circumstances, and determine in the good faith exercise of its discretion whether the transaction is in or is not inconsistent with the best interests of us and our stockholders. We did not previously have a formal policy concerning transactions with related persons.

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Convertible Preferred Stock Financings

October 2012 Series E Preferred Stock Financing.

In October 2012, we entered into a Series E Preferred Stock, Common Stock and Warrant Purchase Agreement, or the Series E Purchase Agreement, pursuant to which we initially issued and sold to investors (i) an aggregate of 48,726,367 shares of Series E redeemable convertible preferred stock at a purchase price of $0.70 per share, for aggregate consideration of $34.1 million, which included the conversion of indebtedness of an aggregate principal amount of $11.5 million plus accrued interest, (ii) an aggregate of 1,437 shares of common stock at a purchase price of $2.40 per share, for aggregate consideration of $3,450 and (iii) warrants to purchase up to 1,058,221 shares of our common stock. In October 2012, the shares of common stock sold in this financing were subject to a reverse split whereby each one hundred shares of common stock converted into one share of common stock. At an additional closing in November 2012, we issued and sold to investors (i) an aggregate of 188,320 additional shares of Series E redeemable convertible preferred stock at a purchase price of $0.70 per share for aggregate additional consideration of $0.1 million and (ii) additional warrants to purchase up to 3,324 shares of our common stock. Upon the closing of this offering, the shares of Series E redeemable convertible preferred stock issued in this financing will convert into 2,038,111 shares of common stock. For a description of the warrants issued by us in connection with the 2012 Series E preferred stock financing, see Description of Capital Stock Warrants .

In connection with the Series E preferred stock financing, we entered into amended and restated investor rights, voting and right of first refusal and co-sale agreements containing registration rights, information rights voting rights and rights of first refusal, among other things, with certain holders of our convertible preferred stock and certain holders of our common stock. These stockholder agreements will terminate upon the closing of this offering, except for the registration rights granted under our sixth amended and restated investor rights agreement dated October 25, 2012 between us and the investors listed therein, or the Investor Rights Agreement, as more fully described below in Description of Capital Stock Registration Rights.

In October 2012, our subsidiary, Ambit Canada, issued and sold to GrowthWorks Canadian Fund Ltd., 2,247,223 Class E shares of Ambit Canada upon the automatic conversion of convertible promissory notes in the aggregate principal amount of $1.5 million plus accrued interest. In January 2013, Ambit Canada entered into a subscription agreement pursuant to which it issued and sold to GrowthWorks Canadian Fund Ltd. an additional 3,916,693 Class E shares at a purchase price of $0.70 per share for aggregate consideration of $2.7 million. In connection with the issuance of the Class E shares of Ambit Canada, Ambit, Ambit Canada and GrowthWorks Canadian Fund Ltd. entered into an amended and restated shareholders agreement containing voting rights, information rights and rights of first refusal, among other things. Ambit, Ambit Canada and GrowthWorks Canadian Fund Ltd. also entered into an amended and restated put agreement whereby GrowthWorks Canadian Fund Ltd. has the right to require that Ambit purchase from it, at any time, its Class C, Series D, Series D-2 and Class E shares in Ambit Canada for cash or for shares of Ambit s Series C-2, Series D, Series D-2 and Series E redeemable convertible preferred stock, respectively. The amended and restated shareholders agreement and the amended and restated put agreement will terminate immediately prior to the closing of this offering when GrowthWorks Canadian Fund Ltd. will be deemed to have exercised the put right and becomes a holder of Ambit s Series C-2, Series D, Series D-2 and Series E redeemable convertible preferred stock. Upon the closing of this offering and following exercise of the GrowthWorks put right, the shares of Series E redeemable convertible preferred stock issued for the shares of Ambit Canada s Class E shares will convert into 256,829 shares of our common stock.

Joseph Regan, one of our directors, is affiliated with GrowthWorks Canadian Fund Ltd.

May 2011 Series D-2 Preferred Stock Financing.

In May 2011, we entered into a Series D-2 and Series D-3 Preferred Stock and Warrant Purchase Agreement pursuant to which we issued and sold to investors (i) an aggregate of 27,762,411 shares of our Series D-2 redeemable convertible preferred stock at a purchase price of $0.70 per share, for aggregate consideration of $19.4 million and (ii) warrants to purchase up to 23,482,828 shares of our Series D-2 redeemable convertible

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preferred stock. In May 2012, the warrants were amended such that the maximum aggregate number of shares of our Series D-2 redeemable convertible preferred stock to be issued upon exercise was 11,741,415 shares. In October 2012 and November 2012, and aggregate of 523,809 of these shares of Series D-2 redeemable convertible preferred stock were converted into common stock at a rate of one share of common stock for each one hundred shares of Series D-2 redeemable convertible preferred stock and warrants to purchase up to 221,532 shares of our Series D-2 redeemable convertible preferred stock were cancelled. Upon the closing of this offering, the remainder of these shares of Series D-2 redeemable convertible preferred stock will convert into 1,134,942 shares of common stock and the remaining warrants will become exercisable for up to 479,987 shares of our common stock. For a description of the warrants issued by us in connection with the Series D-2 preferred stock financing, see Description of Capital Stock Warrants .

In connection with the Series D-2 preferred stock financing, we entered into amended and restated investor rights and right of first refusal and co-sale agreements containing registration rights, information rights and rights of first refusal, among other things, with certain holders of our convertible preferred stock and certain holders of our common stock. These stockholder agreements were superseded by the agreements we entered into in connection with the 2012 Series E preferred stock financing, discussed above.

In May 2011, we and our subsidiary, Ambit Canada, entered into a subscription agreement pursuant to which (i) Ambit Canada issued and sold to GrowthWorks Canadian Fund Ltd., 3,666,169 Series D-2 shares of Ambit Canada at a purchase price of $0.70 per share, for aggregate consideration of $2.6 million and (ii) we issued a warrant to purchase up to 3,101,030 shares of our Series D-2 redeemable convertible preferred stock. In May 2011, this warrant was amended such that the maximum number of shares of our Series D-2 redeemable convertible preferred stock to be issued upon exercise were 1,550,515 shares. Upon the closing of this offering and following exercise of the GrowthWorks put right, the shares of Series D-2 redeemable convertible preferred stock issued for the shares of Ambit Canada s Series D-2 shares will convert into 152,757 shares of our common stock and the remaining warrants will become exercisable for 1,550,515 shares of our common stock. In connection with the issuance by Ambit Canada of its Series D-2 shares, Ambit, Ambit Canada and GrowthWorks Canadian Fund Ltd. entered into an amended and restated shareholders agreement containing voting rights, information rights and rights of first refusal, among other things. Ambit, Ambit Canada and GrowthWorks Canadian Fund Ltd. also entered into an amended and restated put agreement whereby GrowthWorks Canadian Fund Ltd. has the right to require Ambit purchase from it, at any time, the investor s Class C, Series D and Series D-2 shares in Ambit Canada for cash or for shares of Ambit s Series C-2, Series D and Series D-2 redeemable convertible preferred stock, respectively. The shareholders agreement and put agreement were superseded by the agreements entered into among the parties in connection with the 2012 Series E preferred stock financing, discussed above.

Joseph Regan, one of our directors is affiliated with GrowthWorks Canadian Fund Ltd.

The participants in these convertible preferred stock financings included the following holders of more than 5% of our capital stock or entities affiliated with them. The following table presents the number of shares of the applicable series of redeemable convertible preferred stock issued to these related parties in each convertible preferred stock financing:

Participants[1]	Series D-2 Preferred Stock Financing	Series E Preferred Stock Financing
5% or Greater Stockholders		
Apposite Healthcare Fund LP	3,959,589	4,931,751
OrbiMed Private Investments III, LP and its affiliates[2]	3,277,875	6,848,821
Forward Ventures IV, L.P. and its affiliates[3]	3,853,216	3,009,169
Gimv NV and its affiliates[4]	2,255,505	2,501,151
MedImmune Ventures, Inc.	2,772,380	3,661,642
Perseus-Soros Biopharmaceutical Fund, L.P.	5,885,436	4,841,544
Roche Finance Ltd	3,566,093	3,950,669

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(1) Additional detail regarding these stockholders and their equity holdings is provided in Principal Stockholders.
(2) Represents shared purchased by OrbiMed Private Investments III, LP and OrbiMed Associates III, LP.
(3) Represents shared purchased by Forward Ventures IV, L.P. and Forward Ventures IV B, L.P.
(4) Represents shared purchased by Gimv NV and Adviesbeheer Gimv Life Sciences 2004 NV.

Some of our directors are associated with participants in the Series D-2 preferred stock financing and Series E preferred stock financing as indicated in the table below:

Director	Principal Stockholder
David Bonita, M.D.	OrbiMed Private Investments III, LP and its affiliates
Steven A. Elms	Perseus-Soros Biopharmaceutical Fund, L.P.
Standish M. Fleming	Forward Ventures IV, L.P. and its affiliates
Allan P. Marchington, Ph.D.	Apposite Healthcare Fund LP
Isai Peimer	MedImmune Ventures, Inc.

Convertible Note and Warrant Issuances

Bridge Financings

2012 Bridge Financing.

In May 2012 and July 2012, we issued secured subordinated convertible promissory notes in an aggregate amount of $11.5 million to investors including, among others, Apposite Healthcare Fund LP, OrbiMed Private Investments III, LP, Forward Ventures IV, L.P, Gimv NV, MedImmune Ventures, Inc., Perseus-Soros Biopharmaceutical Fund, L.P., Roche Finance Ltd, and their affiliates, each with a maturity date of the earlier of April 30, 2015 or the date of a change of control transaction of Ambit. The notes were secured by a second priority security interest in our assets, excluding intellectual property. In connection with the 2012 bridge financing, Ambit Canada also issued secured subordinated convertible promissory note in May 2012 and July 2012 on substantially the same terms as the notes issued by Ambit in the aggregate amount of $1.5 million to GrowthWorks Canadian Fund Ltd., each with a maturity date of the earlier of April 30, 2015 or the date of a change of control transaction of Ambit.

The promissory notes issued by us in the 2012 bridge financing converted into 17,008,346 shares of our Series E redeemable convertible preferred stock on October 26, 2012 and the promissory notes issued by Ambit Canada in the 2012 bridge financing converted into 2,247,223 shares of Ambit Canada s Class E shares. Upon the closing of this offering, the shares of Series E redeemable convertible preferred stock issued in connection with the conversion of the promissory notes issued by us will convert into 708,681 shares of our common stock and, following exercise of the GrowthWorks put right, which shall occur simultaneously with the conversion of the outstanding shares of our preferred stock into shares of our common stock, the shares of Series E redeemable convertible preferred stock issued for the shares of Ambit Canada s Class E shares issued in connection with the conversion of promissory notes issued by Ambit Canada will convert into 93,634 shares of our common stock.

2010 Bridge Financing.

In September 2010, we issued secured subordinated convertible promissory notes in an aggregate amount of $13.3 million to investors including, among others, Apposite Healthcare Fund LP, OrbiMed Private Investments III, LP, Forward Ventures IV, L.P, Gimv NV, MedImmune Ventures, Inc., Perseus-Soros Biopharmaceutical Fund, L.P., Roche Finance Ltd, and their affiliates, each with a maturity date of January 31, 2012. The notes were secured by a second priority security interest in our assets, excluding intellectual property and assets held for sale. In connection therewith, we also issued warrants to purchase up to an aggregate of 21,826 shares of our common stock to the 2010 bridge financing investors. In connection with the 2010 bridge financing, Ambit Canada also issued a secured promissory note in September 2010 on substantially the same terms as the notes

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issued by Ambit in the aggregate amount of $1.7 million to GrowthWorks Canadian Fund Ltd., with a maturity date of January 31, 2012. In connection therewith, we also issued warrants to purchase shares of our common stock and redeemable convertible preferred stock to GrowthWorks Canadian Fund Ltd.

The promissory notes issued by us in the 2010 bridge financing converted into 23,966,205 shares of our Series D-2 redeemable convertible preferred stock on May 18, 2011. Upon conversion of such promissory notes issued by us, the promissory note issued by Ambit Canada was cancelled and the Series D-2 redeemable convertible preferred stock warrants issued by us to GrowthWorks were automatically exercised for 3,156,967 shares of our Series D-2 redeemable convertible preferred stock. In October 2012 and November 2012, an aggregate of 1,240,068 of these shares of Series D-2 redeemable convertible preferred stock were converted into 516 shares of common stock. Upon the closing of this offering, the remaining shares of Series D-2 redeemable convertible preferred stock issued in connection with the conversion of the promissory notes issued by us and the exercise of the warrants issued by us to GrowthWorks will convert into 1,078,462 shares of common stock and, after giving effect to the warrant exchange, the warrants to purchase our common stock issued by us in connection with the 2010 bridge financing will be exercisable for an aggregate of up to 20,729 shares of common stock. For a description of the warrants issued by us in connection with the 2009 bridge financing, see Description of Capital Stock Warrants .

The participants in the 2010 bridge financing and 2012 bridge financing included the following holders of more than 5% of our capital stock or entities affiliated with them. The following table presents the loan amount provided by each such party in these bridge financings:

Participants[1]	2010 Bridge Financing Loan Amount	2012 Bridge Financing Loan Amount
5% or Greater Stockholders		
Apposite Healthcare Fund LP	$ 1,885,535	$ 1,638,612
OrbiMed Private Investments III, LP and its affiliates[2]	$ 1,133,148	$ 1,384,815
Forward Ventures IV, L.P. and its affiliates[3]	$ 1,834,880	$ 1,594,593
Gimv NV and its affiliates[4]	$ 1,074,059	$ 933,404
MedImmune Ventures, Inc.	$ 1,320,192	$ 1,147,304
Perseus-Soros Biopharmaceutical Fund, L.P.	$ 2,802,613	$ 2,435,594
Roche Finance Ltd	$ 1,698,154	$ 1,475,770
GrowthWorks Canadian Fund Ltd.[5]	$ 1,745,810	$ 1,517,174

(1) Additional detail regarding these stockholders and their equity holdings is provided in Principal Stockholders.
(2) Represents notes held by OrbiMed Private Investments III, LP and OrbiMed Associates III, LP.
(3) Represents notes held by Forward Ventures IV, L.P. and Forward Ventures IV B, L.P.
(4) Represents notes held by Gimv NV and Adviesbeheer Gimv Life Sciences 2004 NV.
(5) Represents amount loaned to Ambit Canada in parallel debt financing.

Some of our directors are associated with participants in the 2010 bridge financing and 2012 bridge financing as indicated in the table below:

Director	Principal Stockholder
David Bonita, M.D.	OrbiMed Private Investments III, LP and its affiliates
Steven A. Elms	Perseus-Soros Biopharmaceutical Fund, L.P.
Standish M. Fleming	Forward Ventures IV, L.P. and its affiliates
Allan P. Marchington, Ph.D.	Apposite Healthcare Fund LP
Isai Peimer	MedImmune Ventures, Inc.
Joseph Regan	GrowthWorks Canadian Fund Ltd.

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In addition, Alexander Zukiwski, M.D., one of our former directors, was associated with MedImmune Ventures, Inc. at the time of the 2010 bridge financing.

Concurrent Private Placement

The investors in the 2012 Series E preferred stock financing have agreed, pursuant to the Series E Purchase Agreement to purchase an aggregate of 1,791,133 shares of our Common Stock, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), in a private placement that will close concurrently with the closing of this offering. The sale of such shares will not be registered under the Securities Act.

Non-Employee Director Agreements

In 2009 we entered into letter agreements with two of our former directors, Dr. Lewis and Mr. Zarrabian. The letter agreement with Dr. Lewis was superseded by the employment agreement we entered into with him in July 2010, when he became our President and Chief Executive Officer. In 2012, we entered into a supplemental letter agreement with Mr. Zarrabian. In 2010 we entered into a letter agreement with one of our directors, Mr. Hasnain, which was restated and superseded in 2011 and amended in 2012. Each of these letter agreements is described in Executive and Director Compensation Non-Employee Director Compensation.

Employment Agreements

We have entered into employment arrangements, with our executive officers, as more fully described in Executive and Director Compensation Employment Agreements, Incentive Compensation and Termination-Based Compensation.

Separation Agreements

We entered into separation agreements with Dr. Lewis, our former president and chief executive officer, and Christopher J. Morl, our former chief operating officer, in October 2011 and January 2012, respectively, as more fully described in Executive and Director Compensation Termination-Based Compensation.

Stock Options Granted to Executive Officers and Directors

We have granted stock options to our executive officers and directors, as more fully described in the section entitled Executive and Director Compensation.

Indemnification Agreements

We have entered into indemnification agreements with each of our directors and executive officers, as described in Executive and Director Compensation Limitation of Liability and Indemnification.

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PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our capital stock outstanding as of March 31, 2013 by:

- Each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- Each of our directors;
- Each of our named executive officers; and
- All of our directors and executive officers as a group.

The number of shares and percentage of shares beneficially owned before the offering shown in the table is based upon 6,453,063 shares of common stock outstanding as of March 31, 2013, which gives effect to (1) the conversion of all outstanding shares of our convertible preferred stock into shares of common stock and (2) the exercise of the put right held by GrowthWorks Canadian Fund Ltd., or the GrowthWorks put right, which shall occur simultaneously with the conversion of the outstanding shares of our preferred stock into shares of our common stock. The number of shares purchased in the concurrent private placement assumes an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). The number of shares and percentage of shares beneficially owned after the offering gives effect to (1) the issuance of 1,791,133 shares of our common stock to certain of our existing stockholders in the concurrent private placement and (2) the issuance by us of 4,645,000 shares of common stock in this offering, in each case assuming an initial public offering price of $14.00 per share (the midpoint of the range set forth on the cover page of this prospectus). The percentage ownership information assumes no exercise of the underwriters over-allotment option.

Each individual or entity shown in the table has furnished information with respect to beneficial ownership. We have determined beneficial ownership in accordance with the SEC s rules. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules include shares of common stock issuable pursuant to the exercise of stock options, warrants or other rights that are either immediately exercisable or exercisable on May 30, 2013, which is 60 days after March 31, 2013. These shares are deemed to be outstanding and beneficially owned by the person holding those options or warrants for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them, subject to applicable community property laws.

Except as otherwise noted below, the address for each person or entity listed in the table is c/o Ambit Biosciences Corporation, 11080 Roselle St., San Diego, California 92121.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned Before Offering	Number of Shares Purchased in Concurrent Private Placement	Number of Shares Beneficially Owned After Offering	Percentage of Shares Beneficially Owned	
				Before Offering	After Offering
5% or greater stockholders:					
Apposite Healthcare Fund LP[1]	1,119,350	246,587	1,365,937	16.8%	10.4%
c/o Ogier Fiduciary Services (Cayman) Ltd 89 Nexus Way Camana Bay, Grand Cayman, KY1-9007, Cayman Islands					
Forward Ventures IV, L.P. and its affiliates[2]	774,918	150,458	925,376	11.9%	7.1%
9255 Towne Centre Drive, Suite 300					

San Diego, CA 92121

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Name and Address of Beneficial Owner	Number of Shares Beneficially Owned Before Offering	Number of Shares Purchased in Concurrent Private Placement	Number of Shares Beneficially Owned After Offering	Percentage of Shares Beneficially Owned Before Offering	Percentage of Shares Beneficially Owned After Offering
Gimv NV and its affiliates[3] Karel Oomsstraat 37 2018 Antwerp, Belgium	575,816	125,056	700,872	8.8%	5.4%
GrowthWorks Canadian Fund Ltd.[4] 2200 130 King Street, West Toronto, ON M5X 3R3	789,048	195,833	984,881	12.1%	7.6%
MedImmune Ventures, Inc.[5] One MedImmune Way Gaithersburg, MD 20878	820,118	183,080	1,003,198	12.4%	7.7%
OrbiMed Private Investments III, LP and its affiliates[6] 767 Third Avenue, 30th Floor New York, NY 10017	1,409,513	342,440	1,751,953	19.9%	13.0%
Perseus-Soros Biopharmaceutical Fund, L.P.[7] 888 Seventh Avenue, 29th Floor New York, NY 10016	1,247,041	242,076	1,489,117	19.0%	11.4%
Roche Finance Ltd[8] Grenzacherstrasse 122 4070 Basel, Switzerland	914,435	197,533	1,111,968	13.8%	8.5%
Directors and named executive officers:					
Michael A. Martino [9]	57,376		57,376	*	*
Alan Fuhrman [10]	12,774		12,774	*	*
Athena Countouriotis, M.D.[11]	12,744		12,744	*	*
David P. Bonita, M.D[12].	1,409,513	342,440	1,751,953	19.9%	13.0%
Steven A. Elms[13]	1,247,041	242,076	1,489,117	19.0%	11.4%
Standish M. Fleming[14]	774,918	150,458	925,376	11.9%	7.1%
Faheem Hasnain [15]	18,611		18,611	*	*
Allan P. Marchington, Ph.D.[16]	1,119,350	246,587	1,365,937	16.8%	10.4%
David R. Parkinson, M.D.					
Isai Peimer					
Joseph Regan					
All current executive officers and directors as a group (11 persons)[17]	4,652,327	981,561	5,633,888	61.2%	40.1%

* Represents beneficial ownership of less than one percent.

(1) Includes (a) 223 shares of common stock held by Apposite Healthcare Fund, LP, or Apposite (b) 895,294 shares of common stock issuable upon conversion of convertible preferred stock held by Apposite, (c) 154,058 shares of common stock issuable upon the exercise of common stock warrants held by Apposite, and (d) 69,775 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by Apposite, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Apposite Healthcare (GP) Limited, the general partner of Apposite, has appointed Apposite Capital LLP as the manager of Apposite. Allan P. Marchington, one of our directors, is a designated member of Apposite Capital LLP and, together with F. David Porter and Stephen Adkin, the other designated members of Apposite Capital LLP, shares voting and

investment control over the shares held by Apposite; however, each disclaims beneficial ownership, except to the extent of their pecuniary interests therein.

(2) Includes (a) 125 shares of common stock held by Forward Ventures IV, L.P., or Forward IV, (b) 641,821 shares of common stock held by Forward IV, issuable upon conversion of convertible preferred stock, (c) 10 shares of common stock held by Forward Ventures IV B, L.P., or Forward IV B, (d) 54,410 shares of

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common stock held by Forward IV B, issuable upon conversion of convertible preferred stock, (e) 6,479 shares of common stock held by Forward Ventures IV-C, L.P., or Forward IV-C, issuable upon conversion of convertible preferred stock, (f) 3,611 shares of common stock held by Forward IV, issuable upon the exercise of common stock warrants held by Forward IV, (g) 306 shares of common stock held by Forward IV B, issuable upon the exercise of common stock warrants held by Forward IV B, (h) 256 shares of common stock held by Forward IV-C, issuable upon the exercise of common stock warrants held by Forward IV-C, (i) 62,594 shares of common stock held by Forward IV, issuable upon the exercise of convertible preferred stock warrants held by Forward IV, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock, and (j) 5,306 shares of common stock held by Forward IV B, issuable upon the exercise of convertible preferred stock warrants held by Forward IV B, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Mr. Fleming, one of our directors, and Dr. Ivor Royston, the managing members of Forward IV Associates, LLC the general partner of Forward IV, Forward IV B and Forward IV-C, and Dr. Stuart Collinson, the key member of Forward IV Associates, LLC, share voting and investment control over the shares held by Forward IV, Forward IV B and Forward IV-C, but disclaim beneficial ownership, except to the extent of their pecuniary interests therein.

(3) Includes (a) 96 shares of common stock held by Gimv NV, (b) 17 shares of common stock held by Adviesbeheer Gimv Life Sciences 2004 NV, or Ad Gimv 2004, (c) 391,313 shares of common stock issuable upon conversion of convertible preferred stock held by Gimv NV, (d) 69,055 shares of common stock issuable upon conversion of convertible preferred stock held by Ad Gimv 2004, (e) 64,252 shares of common stock held by Gimv NV, issuable upon the exercise of common stock warrants held by Gimv NV, (f) 11,338 shares of common stock held by Ad Gimv 2004, issuable upon the exercise of common stock warrants held by Ad Gimv 2004, (g) 33,784 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by Gimv NV assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock, and (h) 5,961 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by Ad Gimv 2004, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Gimv NV is a company listed on NYSE Euronext Brussels. Ad Gimv 2004 is a subsidiary of Gimv NV. Investment and voting control over the shares are exercised by the board of directors of Gimv NV which is comprised of the following 12 members: Herman Daems, Koe Dejonckheere, Leo Victor, Dirk Boogmans, Greet De Leenheer, Christ l Jaris, Jan Kerremans, Sophie Manigart, Martine Reynaers, Eric Spiessens, Emile van der Burg and Bart Van Hooland. Each of these individuals disclaims any beneficial ownership of the shares owned by Gimv NV and Ad Gimv 2004 except to the extent of his or her pecuniary interest in such entity.

(4) Includes (a) 194,249 shares of common stock held by GrowthWorks Canadian Fund Ltd., or GrowthWorks, issuable upon conversion of convertible preferred stock, (b) 530,094 shares of common stock to be issued pursuant to the conversion of convertible preferred stock that GrowthWorks shall receive pursuant to an Amended and Restated Put Agreement between GrowthWorks, us and our Canadian subsidiary, Ambit Biosciences (Canada) Corporation, (c) 101 shares of common stock issuable upon the exercise of common stock warrants held by GrowthWorks and (d) 64,604 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by GrowthWorks, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Voting and investment decisions with respect to the shares held by GrowthWorks are made by its manager GrowthWorks WV Management Ltd. The following are the officers of GrowthWorks WV Management Ltd. who have the authority to authorize such voting and investment decisions on behalf of GrowthWorks: David Levi, president and chief executive officer, Alex Irwin, chief operating officer, and Tim Lee, senior vice president investments. Each member of the group disclaims beneficial ownership of such shares except to the extent of its pecuniary interest therein, if any.

(5) Includes (a) 165 shares of common stock held by MedImmune Ventures, Inc., (b) 635,547 shares of common stock issuable upon conversion of convertible preferred stock held by MedImmune Ventures, Inc., (c) 135,552 shares of common stock issuable upon the exercise of common stock warrants held by MedImmune Ventures, Inc. and (d) 48,854 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by MedImmune Ventures, Inc., assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. MedImmune Ventures, Inc. exercises voting and investment power over the shares held by it. MedImmune Ventures, Inc. is indirectly wholly-owned by AstraZeneca PLC. The shares of AstraZeneca PLC are listed on the New York Stock Exchange as American Depository Shares.

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(6) Includes (a) 307 shares of common stock held by OrbiMed Private Investments III, LP, or OPI III, (b) 2 shares of common stock held by OrbiMed Associates III, LP., or Associates III, (c) 768,866 shares of common stock held by OPI III, issuable upon conversion of convertible preferred stock, (d) 7,322 shares of common stock held by Associates III, issuable upon conversion of convertible preferred stock, (e) 569,829 shares of common stock held by OPI III, issuable upon the exercise of common stock warrants held by OPI III, (f) 5,426 shares of common stock held by Associates III, issuable upon the exercise of common stock warrants held by Associates III, (g) 57,217 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by OPI III, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock, and (h) 544 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by Associates III, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. OrbiMed Capital GP III, LLC, or GP III, is the general partner of OPI III, and OrbiMed Advisors LLC, or Advisors, is the managing member of GP III. Advisors is also the general partner of Associates III. Samuel D. Isaly is the managing member of and owner of a controlling interest in Advisors and may be deemed to have voting and investment power over shares held by OPI III and Associates III. Mr. Isaly disclaims beneficial ownership over such shares, except to the extent of his pecuniary interest therein.

(7) Includes (a) 219 shares of common stock held by Perseus-Soros BioPharmaceutical Fund, LP, or PSBF, (b) 1,122,590 shares of common stock issuable upon conversion of convertible preferred stock held by PSBF, (c) 20,520 shares of common stock issuable upon the exercise of common stock warrants held by PSBF, and (d) 103,712 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by PSBF, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Perseus-Soros Partners, LLC, or PSPGP, is the general partner of PSBF. Aisling Capital LLC, or Aisling Capital, is the managing member of PSPGP. Messrs. Steven A. Elms and Dennis Purcell and Dr. Andrew Schiff are the managing members of Aisling Capital. Each of PSBF, PSPGP, Aisling Capital, Mr. Elms, Mr. Purcell and Dr. Schiff may be deemed to beneficially own the shares held by PSBF. Each of Mr. Elms, Mr. Purcell and Dr. Schiff disclaims any beneficial ownership of the shares owned by PSBF except to the extent of his pecuniary interest in such entity.

(8) Includes (a) 179 shares of common stock held by Roche Finance Ltd, (b) 727,259 shares of common stock issuable upon conversion of convertible preferred stock held by Roche Finance Ltd, (c) 124,156 shares of common stock issuable upon the exercise of common stock warrants held by Roche Finance Ltd, and (d) 62,841 shares of common stock issuable upon the exercise of convertible preferred stock warrants held by Roche Finance Ltd, assuming the adjustment of all outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase shares of our common stock. Roche Finance Ltd exercises voting and investment control over the shares held by it. Roche Finance Ltd is wholly-owned by Roche Holding Ltd. Roche Holding Ltd s American Depository Receipt is cross-listed on OTCQX International Premier under the symbol RHHBY. Roche Holding Ltd s non-voting equity securities and its voting shares are both listed on SIX Swiss Exchange.

(9) Includes 57,376 shares of common stock that Mr. Martino has the right to acquire from us within 60 days of March 31, 2013 pursuant to the exercise of stock options.

(10) Includes 12,693 shares of common stock that Mr. Fuhrman has the right to acquire from us within 60 days of March 31, 2013 pursuant to the exercise of stock options.

(11) Includes 12,744 shares of common stock that Dr. Countouriotis has the right to acquire from us within 60 days of March 31, 2013 pursuant to the exercise of stock options.

(12) Includes the shares of capital stock held by the OrbiMed entities referred to in footnote (6) above. Dr. Bonita, a member of our board, is a Principal at OrbiMed Advisors, the general partner or managing member of the general partner of such OrbiMed entities. Dr. Bonita disclaims any beneficial ownership of the shares held by these entities except to the extent of his pecuniary interest in these entities.

(13) Includes the shares of capital stock held by PSBF referred to in footnote (7) above. Mr. Elms, a member of our board, is a managing member of Aisling Capital, the managing member of the general partner of PSBF. Mr. Elms disclaims any beneficial ownership of the shares held by PSBF except to the extent of his pecuniary interest in such entity.

(14) Includes the shares of capital stock held by the Forward Ventures entities referred to in footnote (2) above. Mr. Fleming, a member of our board, is a managing member of Forward IV Associates, LLC, the general partner of such Forward Ventures entities. Voting and investment control over such shares is shared between Mr. Fleming, Dr. Ivor Royston, a managing member of Forward IV Associates, LLC and Dr. Stuart

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Collinson, a key member of Forward IV Associates, LLC. Each of Mr. Fleming and Drs. Royston and Collinson disclaims any beneficial ownership of the shares held by these entities except to the extent of his pecuniary interest in these entities.

(15) Includes 18,611 shares of common stock that Mr. Hasnain has the right to acquire from us within 60 days of December 31, 2012 pursuant to the exercise of stock options.

(16) Includes the shares of capital stock held by Apposite referred to in footnote (1) above. Dr. Marchington, a member of our board, has been designated by Apposite Capital LLP, the manager of Apposite, together with F. David Porter and Stephen Adkin, the other designated members of Apposite Capital LLP, to exercise shared voting and investment control over the shares held by Apposite. Each of Dr. Marchington, Mr. Porter and Mr. Adkin disclaims any beneficial ownership of the shares held by Apposite except to the extent of his pecuniary interest therein.

(17) Includes the shares of capital stock referred to in footnotes (9), (10), (11), (12), (13), (14), (15) and (16).

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DESCRIPTION OF CAPITAL STOCK

Upon the closing of this offering and the filing of our amended and restated certificate of incorporation, our authorized capital stock will consist of 200,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 shares of preferred stock, par value $0.001 per share.

As of March 31, 2013, we had outstanding 3,990 shares of our common stock, held by 91 stockholders of record. This amount excludes the conversion of all outstanding shares of our convertible preferred stock prior to the closing of this offering (including 1,538,461 shares of our Series C-2 redeemable convertible preferred stock, 612,649 shares of Series D redeemable convertible preferred stock, 3,666,169 shares of our Series D-2 redeemable convertible preferred stock and 6,163,916 shares of our Series E redeemable convertible preferred stock to be issued pursuant to the exercise of the GrowthWorks put right).

Based on the number of shares of common stock outstanding as of March 31, 2013, and assuming (1) the conversion of all outstanding shares of our convertible preferred stock (including the shares to be issued pursuant to the exercise of the GrowthWorks put right), (2) the issuance and sale by us of 1,791,133 shares of common stock in the concurrent private placement to certain of our existing stockholders assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), and (3) the issuance by us of 4,645,000 shares of common stock in this offering assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), there will be 12,889,196 shares of common stock outstanding upon completion of this offering and the concurrent private placement.

As of March 31, 2013, there were 1,214,212 shares of common stock subject to outstanding options under our 2011 pre-IPO plan, 1,155,322 shares of common stock subject to outstanding warrants and 15,491,926 shares of convertible preferred stock subject to outstanding warrants.

The following description of our capital stock and provisions of our amended and restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the amended and restated certificate of incorporation and the amended and restated bylaws that will be in effect upon the closing of this offering. Copies of these documents have been filed with the SEC as exhibits to our registration statement, of which this prospectus forms a part.

Common Stock

Voting Rights. Each holder of common stock is entitled to one vote for each share of common stock on all matters submitted to a vote of the stockholders, including the election of directors. Our amended and restated certificate of incorporation and amended and restated bylaws do not provide for cumulative voting rights. Because of this, the holders of a majority of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

Dividends. Subject to preferences that may be applicable to any then outstanding convertible preferred stock, the holders of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

Liquidation. In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of convertible preferred stock.

Rights and Preferences. Upon effectiveness of this registration statement, holders of common stock will have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions

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applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of convertible preferred stock that we may designate and issue in the future.

Fully Paid and Nonassessable. All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, duly authorized, validly issued, fully paid and nonassessable.

Convertible Preferred Stock

On March 31, 2013, there were 135,397,633 shares of convertible preferred stock outstanding (assuming the exercise of the GrowthWorks put right), held of record by 24 stockholders. Upon the closing of this offering, all outstanding shares of convertible preferred stock will have been converted into 6,449,073 shares of our common stock. Upon the closing of this offering, our board of directors will have the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences, privileges and restrictions of the shares of each wholly unissued series, including dividend rights, conversion rights, voting rights, terms of redemption, liquidation preference and sinking fund terms, and to increase or decrease the number of shares of any such series (but not below the number of shares of such series then outstanding).

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could have the effect of restricting dividends on our common stock, diluting the voting power of our common stock, impairing the liquidation rights of our common stock or otherwise adversely affect the rights of holders of our common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change of control and may adversely affect the market price of the common stock. Upon the closing of this offering, no shares of preferred stock will be outstanding, and we have no current plans to issue any shares of preferred stock.

Warrants

As of March 31, 2013, there were outstanding warrants to purchase the following shares of our capital stock:

Description	# of Shares of Common Stock After this Offering	Weighted-Average Exercise Price After this Offering
Common Stock	1,155,322	$ 2.53
Series C redeemable convertible preferred stock	15,075	103.26
Series D redeemable convertible preferred stock	218	54.99
Series D-2 redeemable convertible preferred stock[(1)]	630,305	2.31

(1) Assumes the warrants described in item (h) below are exercisable for Series D-2 redeemable convertible preferred stock.

The following list sets forth information regarding all outstanding warrants to purchase our capital stock:

(a) In October 2005, in connection with a venture loan with Horizon Technology Funding Company II LLC and Horizon Technology Funding Company III, LLC, we issued warrants to purchase an aggregate of 232,558 shares of our Series C redeemable convertible preferred stock, with an initial exercise price of $4.30 per share. Upon the closing of this offering, these warrants will be exercisable for 9,689 shares of common stock at an exercise price of $103.26 per share. These warrants terminate in October 2015.

(b) In October 2005, December 2005, July 2006, October 2006, December 2006, March 2007 and June 2007, in connection with borrowing under an equipment line of credit with Oxford Finance

Corporation, we issued warrants to purchase an aggregate of 26,219 shares of our Series C redeemable convertible preferred stock, with an initial exercise price of $4.30 per share. Upon the closing of this offering, these warrants will be exercisable for 1,092 shares of common stock at an exercise price of $103.26 per share. These warrants terminate eight years after the date issued.

(c) In July 2006, October 2006, December 2006, March 2007 and June 2007, in connection with borrowing under an equipment line of credit with Webster Bank, National Association, we issued warrants to purchase an aggregate of 10,217 shares of our Series C redeemable convertible preferred stock, with an initial exercise price of $4.30 per share. Upon the closing of this offering, these warrants will be exercisable for 425 shares of common stock at an exercise price of $103.26 per share. These warrants terminate eight years after the date issued.

(d) In September 2007, in connection with a venture loan with Horizon Technology Funding Company V LLC, we issued a warrant to purchase an aggregate of 93,023 shares of our Series C redeemable convertible preferred stock, with an initial exercise price of $4.30 per share. Upon the closing of this offering, this warrant will be exercisable for 3,875 shares of common stock at an exercise price of $103.26 per share. This warrant terminates in September 2017.

(e) In August 2008, in connection with borrowing under an equipment line of credit with Oxford Finance Corporation, we issued a warrant to purchase an aggregate of 2,369 shares of our Series D redeemable convertible preferred stock, with an initial exercise price of $5.06 per share. Upon the closing of this offering, this warrant will be exercisable for 218 shares of common stock at an exercise price of $54.99 per share. This warrant terminates in August, 2016.

(f) On multiple dates in 2009, in connection with our 2009 bridge financing, we issued warrants to purchase shares of our common stock. Certain of these warrants were cancelled in connection with the warrant exchange described in item (m) below. The remaining of these warrants are exercisable for an aggregate of 78 shares of our common stock at an exercise price of $2,184.00 per share. These warrants terminate 10 years after the date issued.

(g) In July, September and November 2009, in connection with Ambit Canada s bridge financing, we issued warrants to purchase preferred stock to GrowthWorks with an exercise price of $5.06 per share. These warrants were automatically exercised for shares of our Series D redeemable convertible preferred stock in connection with the conversion of the convertible promissory notes issued by us in the 2009 bridge financing.

(h) In March 2010, in connection with a venture loan with Compass Horizon Funding Company LLC and Oxford Finance Corporation, we issued a warrant to each of Horizon and Oxford to purchase up to 142,292 shares of our Series D redeemable convertible preferred stock or, at the option of the holders of the warrants, up to 1,028,571 shares of our Series D-2 redeemable convertible preferred stock. The exercise price of these warrants is $5.06 per share if exercised for Series D redeemable convertible preferred stock or $0.70 per share if exercised for Series D-2 redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be exercisable for an aggregate of 85,714 shares of our common stock at an exercise price of $16.80 per share. These warrants terminate 10 years after the date issued.

(i) In September 2010, in connection with our 2010 bridge financing, we issued warrants to purchase shares of our common stock. Certain of these warrants were cancelled in connection with the warrant exchange described in item (m) below. The remaining of these warrants are exercisable for an aggregate of 40 shares of our common stock at an exercise price of $3,696.00 per share. These warrants terminate 10 years after the date issued.

(j) In September 2010, in connection with Ambit Canada s bridge financing, we issued warrants to purchase preferred stock or common stock to GrowthWorks. These warrants were automatically exercised for shares of our Series D-2 redeemable convertible preferred stock in connection with the conversion of the convertible promissory notes issued by us in the 2010 bridge financing.

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(k) In May 2011, in connection with our Series D-2 preferred stock financing, we issued warrants to purchase shares of our Series D-2 redeemable convertible preferred stock, with an initial exercise price of $0.001 per share. Certain of these warrants were cancelled in connection with the 2012 Series E Financing. The remaining of these warrants are exercisable for an aggregate of 13,070,398 shares of our Series D-2 redeemable convertible preferred stock. Upon the closing of this offering, these remaining warrants will be exercisable for an aggregate of 544,591 shares of our common stock at an exercise price of $0.024 per share. These warrants terminate 10 years after the date issued.

(l) In October and November 2012, in connection with our Series E preferred stock financing, we issued warrants to purchase shares of our common stock. These warrants are exercisable for an aggregate of 1,061,545 shares of our common stock at an exercise price of $0.24 per share. These warrants terminate 10 years after the date issued.

(m) In November 2012 and December 2012, in connection with the Series E preferred stock financing, warrants to purchase an aggregate of up to 729 shares of our common stock held by certain qualified investors were exchanged or became exchangeable for warrants to purchase an aggregate of up to 72,970 shares of our common stock with an exercise price of $21.84 per share These warrants terminate 10 years after the date that the applicable cancelled warrant was issued. Also in November 2012 and December 2012, in connection with the Series E preferred stock financing, warrants to purchase an aggregate of up to 206 shares of our common stock held by certain qualified investors were exchanged or became exchangeable for warrants to purchase an aggregate of up to 20,690 shares of our common stock with an exercise price of $36.96 per share. These warrants terminate 10 years after the date that the applicable cancelled warrant was issued.

Each of our warrants contains provisions for the adjustment of the exercise price and the aggregate number of shares issuable upon the exercise of the warrant in the event of stock dividends, stock splits, reorganizations, reclassifications and consolidations. Each of our warrants also contains a net exercise provision under which the holder may, in lieu of payment of the exercise price in cash, surrender the warrant and receive a net amount of shares based on the fair market value of the underlying stock at the time of exercise of the warrant after deduction of the aggregate exercise price.

Registration Rights

Common and Convertible Preferred Stock

According to the terms of our Investor Rights Agreement, certain of our stockholders are entitled to demand, Form S-3 and piggyback registration rights. These stockholders will hold an aggregate of 5,005,963 shares eligible for registration under the Investor Rights Agreement, or approximately 38.8%, of our total outstanding common stock, upon the closing of this offering and the concurrent private placement and the conversion of all existing series of our convertible preferred stock into shares of our common stock, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). Such stockholders have agreed not to exercise their registration rights during the lock-up period for this offering. See Shares Eligible for Future Sale Lock-up Agreements.

Demand Registration Rights. At any time beginning 180 days following the effective date of this registration statement, the holders of at least a majority of the registrable securities, as defined under the Investor Rights Agreement, have the right to make up to three demands that we file a registration statement to register all or a portion of their shares so long as the aggregate offering price of securities requested to be sold under such registration statement is at least $25,000,000, net of underwriting discounts and commissions and subject to specified exceptions.

Form S-3 Registration Rights. If we are eligible to file a registration statement on Form S-3, one or more holders of registrable securities has the right to demand that we file up to six registration statements on Form S-3 so long as the aggregate offering price of the securities to be sold under the registration statement on Form S-3 is at least $1,000,000, net of underwriting discounts and commissions, and subject to specified exceptions.

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Piggyback Registration Rights. If we register any securities for public sale, holders of registrable securities are entitled to written notice of the registration and will have the right to include their shares in the registration statement. The underwriters of any offering will have the right to limit the number of shares having registration rights to be included in the registration statement, but not below 25% of the total number of shares included in the registration statement, unless such offering is our initial public offering and such registration does not include shares of any other selling stockholders, in which case any and all shares held by selling stockholders may be excluded from the offering.

Expenses of Registration; Indemnification. Generally, we are required to bear all registration expenses incurred in connection with the demand, Form S-3 and piggyback registrations described above, other than underwriting discounts and commissions. The Investor Rights Agreement contains customary indemnification provisions with respect to registration rights.

Expiration of Registration Rights. The demand, Form S-3 and piggyback registration rights discussed above will terminate five years after the closing of this offering. In addition, the registration rights of a holder of registrable securities will expire if all of the holder s registrable securities may be sold in a single 90-day period under Rule 144 of the Securities Act.

Delaware Anti-Takeover Law and Provisions of Our Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Our amended and restated certificate of incorporation and our amended and restated bylaws, which will become effective upon the closing of this offering, contain certain provisions that could have the effect of delaying, deterring or preventing another party from acquiring control of us, and therefore could adversely affect the market price of our common stock. These provisions and certain provisions of Delaware General Corporation Law, or DGCL, which are summarized below, may also discourage coercive takeover practices and inadequate takeover bids, and are designed, in part, to encourage persons seeking to acquire control of us to negotiate first with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate more favorable terms with an unfriendly or unsolicited acquirer outweigh the disadvantages of potentially discouraging a proposal to acquire us.

Delaware Anti-Takeover Law

We are subject to Section 203 of the DGCL. Section 203 generally prohibits a public Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years after the date of the transaction in which the person became an interested stockholder, unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the number of shares outstanding (a) shares owned by persons who are directors and also officers and (b) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or subsequent to the date of the transaction, the business combination is approved by the board and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;

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any sale, transfer, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;

subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and

the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as an entity or person who, together with the person s affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Amended and Restated Certificate of Incorporation and Bylaws

Among other things, our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the closing of this offering:

permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change of control);

provide that the authorized number of directors may be changed only by resolution of the board of directors;

provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;

divide our board of directors into three classes;

require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;

provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder s notice;

do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election); and

provide that special meetings of our stockholders may be called only by the chairman of the board, our chief executive officer or by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors.

The amendment of any of these provisions would require approval by the holders of at least 66 2/3% of our then outstanding common stock.

The provisions of the DGCL and the provisions of our amended and restated certificate of incorporation and amended and restated bylaws, as amended upon the closing of this offering, could have the effect of discouraging others from attempting hostile takeovers and, as a consequence, they might also inhibit temporary fluctuations in the market price of our common stock that often result from actual or rumored hostile takeover attempts. These provisions might also have the effect of preventing changes in our management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders might otherwise deem to be in their best interests.

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Listing on the Nasdaq Global Market

We have applied for listing on the Nasdaq Global Market under the symbol AMBI .

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC, located at 6201 15th Avenue, Brooklyn, NY 11219.

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SHARES ELIGIBLE FOR FUTURE SALE

Immediately prior to this offering, there has been no public market for our common stock. Future sales of substantial amounts of common stock in the public market could adversely affect prevailing market prices. Furthermore, since only a limited number of shares will be available for sale shortly after this offering because of contractual and legal restrictions on resale described below, sales of substantial amounts of common stock in the public market after the restrictions lapse could adversely affect the prevailing market price for our common stock as well as our ability to raise equity capital in the future.

Based on the number of shares of common stock outstanding as of March 31, 2013, upon the closing of this offering and the concurrent private placement, 12,889,196 shares of common stock will be outstanding, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover of this prospectus) assuming no exercise of the underwriters over-allotment option and no exercise of options or stock warrants. All of the shares sold in this offering will be freely tradable unless held by an affiliate of ours. Except as set forth below, the remaining 8,244,196 shares of common stock outstanding after this offering, which includes the shares issued in the concurrent private placement, will be restricted as a result of securities laws or lock-up agreements. These remaining shares will generally become available for sale in the public market as follows:

- no restricted shares will be eligible for immediate sale upon the closing of this offering;
- up to 6,282,022 restricted shares will be eligible for sale under Rule 144 or Rule 701 upon expiration of lock-up agreements at least 180 days after the date of this offering assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus); and
- the remainder of the restricted shares will be eligible for sale from time to time thereafter upon expiration of their respective one-year holding periods under Rule 144, but could be sold earlier if the holders exercise any available registration rights.

Rule 144

In general, under Rule 144 as currently in effect, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, any person who is not an affiliate of ours and who has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, provided current public information about us is available. In addition, under Rule 144, any person who is not an affiliate of ours and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available. Beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is an affiliate of ours and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of restricted shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after this offering; or
- the average weekly trading volume of our common stock on the Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Sales of restricted shares under Rule 144 held by our affiliates or persons selling shares on behalf of our affiliates are also subject to requirements regarding the manner of sale, notice and the availability of current public information about us. Rule 144 also provides that affiliates relying on Rule 144 to sell shares of our common stock that are not restricted shares must nonetheless comply with the same restrictions applicable to restricted shares, other than the holding period requirement.

Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted shares have entered into lock-up agreements as described below and their restricted shares will become eligible for sale at the expiration of the restrictions set forth in those agreements.

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Rule 701

Under Rule 701, shares of our common stock acquired upon the exercise of currently outstanding options or pursuant to other rights granted under our stock plans may be resold, by:

- persons other than affiliates, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, subject only to the manner-of-sale provisions of Rule 144; and
- our affiliates, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, subject to the manner-of-sale and volume limitations, current public information and filing requirements of Rule 144, in each case, without compliance with the six-month holding period requirement of Rule 144.

As of March 31, 2013, options to purchase a total of 1,214,212 shares of common stock were outstanding, of which 112,381 were vested. Of the total number of shares of our common stock issuable under these options, all are subject to contractual lock-up agreements with us or the underwriters described below under Underwriting and will become eligible for sale at the expiration of those agreements.

Lock-up Agreements

We, our directors and executive officers, and substantially all of our other stockholders and optionholders have agreed, without the prior written consent of Citigroup Global Markets Inc. and Leerink Swann LLC, not to offer, sell, issue (in the case of us), contract to sell, pledge or, among other things, dispose of, directly or indirectly, any shares of our capital stock or any securities convertible into, or exercisable or exchangeable for such capital stock for a period of 180 days after the date of this prospectus. The lock-up agreements contain certain exceptions, including, among others, allowing stockholders to (1) dispose of shares of common stock purchased in open market transactions after the completion of this offering, provided that no filing by any party under the Securities Exchange Act of 1934, as amended, shall be required or shall be voluntarily made in connection with subsequent sales of common stock acquired in such open market transactions, and (2) enter into or amend a Rule 10b5-1 sales plan if then permitted by us and applicable law, provided that during the 180-day lock-up period (a) no shares subject to the Rule 10b5-1 sales plan may be sold and (b) neither we, the other lock-up participants nor any of our representatives may publicly disclose the establishment of such a plan. In addition, we are permitted, among other specified exceptions, to offer, issue and sell shares of common stock to one or more counterparties in connection with any acquisition or strategic investment (including any joint venture, strategic alliance or partnership), provided that the aggregate number of shares of common stock issued shall not exceed 10% of the number of shares of common stock outstanding after this offering any such counterparty agrees to the foregoing lock-up restrictions for the remainder of the 180-day lock-up period.

Registration Rights

Upon the closing of this offering, the holders of 6,806,883 shares of our common stock and warrants to purchase up to shares of our common stock will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up arrangement described above and assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates, immediately upon the effectiveness of the registration statement of which this prospectus is a part. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock. See Description of Capital Stock Registration Rights.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act after the closing of this offering to register the shares of our common stock that are issuable pursuant to our 2011 pre-IPO plan, 2013 post-IPO plan and ESPP. The registration statement is expected to be filed and become effective as soon as practicable after the closing of this offering. Accordingly, shares registered under the registration statement will be available for sale in the open market following its effective date, subject to vesting of such shares, Rule 144 volume limitations and the lock-up agreements described above, if applicable.

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MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following summary describes the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income taxes and does not deal with state, local or non-U.S. tax consequences that may be relevant to Non-U.S. Holders in light of their particular circumstances, nor does it address U.S. federal tax consequences other than income taxes. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Internal Revenue Code of 1986, as amended, or the Code, such as banks, thrifts, financial institutions, insurance companies, tax-exempt organizations, broker-dealers and traders in securities, U.S. expatriates, controlled foreign corporations, passive foreign investment companies, corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or integrated investment or other risk reduction strategy, tax-qualified retirement plans, persons deemed to sell our common stock under the constructive sale provisions of the Code, persons subject to the alternative minimum tax, persons that own, or are deemed to own, more than 5% of our outstanding common stock (except to the extent specifically set forth below), partnerships and other pass-through entities, and investors or partners in such pass-through entities or an entity that is treated as a disregarded entity for U.S. federal income tax purposes (regardless of its place of organization or formation). Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, the Treasury regulations promulgated thereunder, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment).

The following discussion is for general information only and is not tax advice. Persons considering the purchase of our common stock pursuant to this offering should consult their own tax advisors concerning the U.S. federal income tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local or non-U.S. tax consequences or any U.S. federal non-income tax consequences.

For the purposes of this discussion, a Non-U.S. Holder is, for U.S. federal income tax purposes, a beneficial owner of common stock that is not a U.S. Holder. A U.S. Holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the United States, (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person. Also, partnerships, or other entities that are treated as partnerships for U.S. federal income tax purposes (regardless of their place of organization or formation) and partners in such partnerships or other entities and entities that are treated as disregarded entities for U.S. federal income tax purposes (regardless of their place of organization or formation) are not addressed by this discussion and are, therefore, not considered to be Non-U.S. Holders for the purposes of this discussion.

Distributions

Subject to the discussion below, distributions, if any, made on our common stock to a Non-U.S. Holder of our common stock to the extent made out of our current or accumulated earnings and profits (as determined

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under U.S. federal income tax principles) generally will constitute dividends for U.S. tax purposes and will be subject to withholding tax at a rate of 30% of the gross amount of the dividends or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide us or our paying agent with a properly executed IRS Form W-8BEN, or other appropriate form, certifying the Non-U.S. Holder s entitlement to benefits under that treaty. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder s behalf, the Non-U.S. Holder will be required to provide appropriate documentation to such agent. The Non-U.S. Holder s agent will then be required to provide certification to us or our paying agent, either directly or through other intermediaries. This certification must be provided to us or the relevant paying agent prior to the payment of dividends and must be updated periodically. If you do not provide the relevant paying agent with the required certification but are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you should consult with your own tax advisor to determine if you are able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the Non-U.S. Holder s conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that such Non-U.S. Holder maintains in the United States) if a properly executed IRS Form W-8ECI (or applicable successor form), stating that the dividends are so connected, is furnished to us or our paying agent (or, if stock is held through a financial institution or other agent, to such agent). In general, such effectively connected dividends will be subject to U.S. federal income tax, on a net income basis at the regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional branch profits tax, which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder s effectively connected earnings and profits, subject to certain adjustments. Non-U.S. Holders are urged to consult any applicable income tax treaties that may provide for different rules.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will first reduce your adjusted basis in our common stock as a non-taxable return of capital, but not below zero, and then will be treated as capital gain and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section.

Gain On Disposition of Our Common Stock

Subject to the discussion below under Recently Enacted Legislation Affecting Taxation of Our Common Stock Held By Or Through Non-U.S. Entities, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless (a) the gain is effectively connected with a trade or business of such Non-U.S. Holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that such Non-U.S. Holder maintains in the United States), (b) the Non-U.S. Holder is a nonresident alien individual and is present in the United States for 183 or more days in the taxable year of the disposition and certain other conditions are met, or (c) our common stock constitutes a United States real property interest by reason of our status as a United States real property holding corporation within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such Non-U.S. Holder s holding period.

If you are a Non-U.S. Holder described in (a) above, you will be required to pay tax on the net gain derived from the sale at regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies, and corporate Non-U.S. Holders described in (a) above may be subject to the additional branch profits tax at a 30% rate (or such lower rate as may be specified by an applicable income tax treaty) on its effectively connected earnings and profits, subject to certain adjustments. If you are an individual Non-U.S. Holder described in (b) above, you will be required to pay a flat 30% tax (or such lower rate specified by an applicable income tax treaty) on the gain derived from the sale, which gain may be offset by U.S. source capital losses (even though

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you are not considered a resident of the United States). With respect to (c) above, in general, we would be a United States real property holding corporation if interests in U.S. real estate comprised (by fair market value) at least half of our assets. We believe that we are not, and do not anticipate becoming, a United States real property holding corporation, however, there can be no assurance that we will not become a U.S. real property holding corporation in the future. Even if we are treated as a U.S. real property holding corporation, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder s holding period and (2) our common stock is regularly traded, as defined in applicable Treasury regulations, on an established securities market. We expect our common stock to be regularly traded on an established securities market, but there can be no assurance that our common stock will be so traded. If gain on the sale or other taxable disposition of our stock were subject to taxation as described in (c) above, the Non-U.S. Holder would be subject to regular U.S. federal income tax with respect to such gain in generally the same manner as a U.S. person.

Information Reporting Requirements and Backup Withholding

Generally, we or certain financial middlemen must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the Non-U.S. Holder to whom any such dividends are paid. These information reporting requirements apply even if no withholding was required because the distributions were effectively connected with the Non-U.S. Holder s conduct of a United States trade or business, or withholding was reduced or eliminated by an applicable income tax treaty. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient s country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or IRS Form W- 8ECI or otherwise establishes an exemption. Notwithstanding the foregoing, backup withholding may apply if the relevant paying agent has actual knowledge, or reason to know, that the holder is a U.S. person who is not an exempt recipient. The current backup withholding rate is 28%.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or non-U.S., except that information reporting and such requirements may be avoided if the holder provides a properly executed IRS Form W-8BEN or otherwise meets documentary evidence requirements for establishing Non-U.S. Holder status or otherwise establishes an exemption. Except as described in the discussion of recently enacted legislation below, U.S. information reporting and backup withholding requirements will generally not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. Information reporting and backup withholding requirements may, however, apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is, in fact, a U.S. person. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

Backup withholding is not an additional tax. If backup withholding is applied to you, you should consult with your own tax advisor to determine if you are able to obtain a tax benefit or credit with respect to such backup withholding.

Recently Enacted Legislation Affecting Taxation of Our Common Stock Held By Or Through Non-U.S. Entities

Recently enacted legislation may impose withholding taxes on certain types of payments made to foreign financial institutions (as specially defined under those rules) and certain other non-U.S. entities. Under this

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legislation, the failure to comply with additional certification, information reporting and other specified requirements could result in withholding tax being imposed on payments of dividends and sales proceeds to foreign intermediaries or certain Non-U.S. Holders.

The legislation imposes a 30% withholding tax on dividends on, and gross proceeds from the sale or other disposition of, our common stock paid to a foreign financial institution or to a foreign non-financial entity, unless (i) the foreign financial institution undertakes certain diligence and reporting obligations (ii) the foreign non-financial entity either certifies it does not have any substantial U.S. owners or furnishes identifying information regarding each substantial U.S. owner, or (iii) the foreign financial institution or foreign non-financial entity otherwise qualifies for an exemption from these rules. In addition, if the payee is a foreign financial institution and is subject to the diligence and reporting requirements in clause (i) above, it generally must enter into an agreement with the U.S. Treasury Department that requires, among other things, that it undertake to identify accounts held by certain U.S. persons or U.S.-owned foreign entities, annually report certain information about such accounts, and withhold 30% on payments to non-compliant foreign financial institutions and certain other account holders. Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing this legislation may be subject to different rules.

Under applicable Treasury regulations, any obligation to withhold under the new legislation with respect to dividends on our common stock will not begin until January 1, 2014, and with respect to gross proceeds on disposition of our common stock, will not begin until January 1, 2017. Because we may not know the extent to which a distribution is a dividend for U.S. federal income tax purposes as the time it is made, for purposes of these withholding rules, we may treat the entire distribution as a dividend.

Non-U.S. Holders of our common stock should consult their tax advisors regarding the effect, if any, of this legislation on their ownership and disposition of our common stock.

THE PRECEDING DISCUSSION OF MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS IS FOR GENERAL INFORMATION ONLY. IT IS NOT TAX ADVICE. EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW, AS WELL AS TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, NON-U.S. OR U.S. FEDERAL NON-INCOME TAX LAWS.

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UNDERWRITING

Citigroup Global Markets Inc. and Leerink Swann LLC are acting as joint book-running managers of the offering and as representatives of the underwriters named below. Subject to the terms and conditions stated in the underwriting agreement dated the date of this prospectus, each underwriter named below has severally agreed to purchase, and we have agreed to sell to that underwriter, the number of shares set forth opposite the underwriter s name.

Underwriter	Number of Shares
Citigroup Global Markets Inc.	
Leerink Swann LLC	
BMO Capital Markets Corp.	
Robert W. Baird & Co. Incorporated	
Total	4,645,000

The underwriting agreement provides that the obligations of the underwriters to purchase the shares included in this offering are subject to approval of legal matters by counsel and to other conditions. The underwriters are obligated to purchase all the shares (other than those covered by the over-allotment option described below) if they purchase any of the shares.

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount from the initial public offering price not to exceed $ per share. If all the shares are not sold at the initial offering price, the underwriters may change the offering price and the other selling terms. The representatives have advised us that the underwriters do not intend to make sales to discretionary accounts.

If the underwriters sell more shares than the total number set forth in the table above, we have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to additional shares at the public offering price less the underwriting discount. The underwriters may exercise the option solely for the purpose of covering over-allotments, if any, in connection with this offering. To the extent the option is exercised, each underwriter must purchase a number of additional shares approximately proportionate to that underwriter s initial purchase commitment. Any shares issued or sold under the option will be issued and sold on the same terms and conditions as the other shares that are the subject of this offering.

We, our directors and executive officers, and substantially all of our other stockholders and optionholders have agreed, without the prior written consent of Citigroup Global Markets Inc. and Leerink Swann LLC, not to offer, sell, issue (in the case of us), contract to sell, pledge or, among other things, dispose of, directly or indirectly, any shares of our capital stock or any securities convertible into, or exercisable or exchangeable for such capital stock for a period of 180 days after the date of this prospectus. Citigroup Global Markets Inc. and Leerink Swann LLC in their sole discretion may release any of the securities subject to these lock-up agreements at any time which, in the case of directors and executive officers, shall be with notice. The lock-up agreements contain certain exceptions, including, among others, allowing stockholders to (1) dispose of shares of common stock purchased in open market transactions after the completion of this offering, provided that no filing by any party under the Securities Exchange Act of 1934, as amended, shall be required or shall be voluntarily made in connection with subsequent sales of common stock acquired in such open market transactions, and (2) enter into or amend a Rule 10b5-1 sales plan if then permitted by us and applicable law, provided that during the 180-day lock-up period (a) no shares subject to the Rule 10b5-1 sales plan may be sold and (b) neither we, the other lock-up participants nor any of our representatives may publicly disclose the establishment of such a plan. In addition, we are permitted, among other specified exceptions, to offer, issue and sell shares of common stock to one or more counterparties in connection with any acquisition or strategic investment (including any joint

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venture, strategic alliance or partnership), provided that the aggregate number of shares of common stock issued shall not exceed 10% of the number of shares of common stock outstanding after this offering and such counterparty agrees to the foregoing lock-up restrictions for the remainder of the 180-day lock-up period.

Prior to this offering, there has been no public market for our shares. Consequently, the initial public offering price for the shares was determined by negotiations between us and the representatives. Among the factors considered in determining the initial public offering price were our results of operations, our current financial condition, our future prospects, our markets, the economic conditions in and future prospects for the industry in which we compete, our management, and currently prevailing general conditions in the equity securities markets, including current market valuations of publicly traded companies considered comparable to our company. We cannot assure you, however, that the price at which the shares will sell in the public market after this offering will not be lower than the initial public offering price or that an active trading market in our shares will develop and continue after this offering.

We have applied to have our shares listed on the Nasdaq Global Market under the symbol AMBI.

The following table shows the underwriting discounts and commissions that we are to pay to the underwriters in connection with this offering. These amounts are shown assuming both no exercise and full exercise of the underwriters over-allotment option.

	Paid by Ambit Biosciences Corporation	
	No Exercise	**Full Exercise**
Per Share	$	$
Total	$	$

We estimate that our portion of the total expenses of this offering will be $2.85 million.

We have also agreed to reimburse the underwriters for certain of their expenses in an amount up to $30,000 as set forth in the underwriting agreement.

In connection with the offering, the underwriters may purchase and sell shares in the open market. Purchases and sales in the open market may include short sales, purchases to cover short positions, which may include purchases pursuant to the over-allotment option, and stabilizing purchases.

Short sales involve secondary market sales by the underwriters of a greater number of shares than they are required to purchase in the offering.

Covered short sales are sales of shares in an amount up to the number of shares represented by the underwriters over-allotment option.

Naked short sales are sales of shares in an amount in excess of the number of shares represented by the underwriters over-allotment option.

Covering transactions involve purchases of shares either pursuant to the underwriters over-allotment option or in the open market in order to cover short positions.

To close a naked short position, the underwriters must purchase shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares in the open market after pricing that could adversely affect investors who purchase in the offering.

To close a covered short position, the underwriters must purchase shares in the open market or must exercise the over-allotment option. In determining the source of shares to close the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the over-allotment option.

Stabilizing transactions involve bids to purchase shares so long as the stabilizing bids do not exceed a specified maximum.

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Purchases to cover short positions and stabilizing purchases, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of the shares. They may also cause the price of the shares to be higher than the price that would otherwise exist in the open market in the absence of these transactions. The underwriters may conduct these transactions on the Nasdaq Global Market, in the over-the-counter market or otherwise. If the underwriters commence any of these transactions, they may discontinue them at any time.

Affiliations

The underwriters are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, principal investment, hedging, financing and brokerage activities. The underwriters and their respective affiliates may, from time to time, engage in transactions with and perform services for us in the ordinary course of their business for which they may receive customary fees and reimbursement of expenses. In the ordinary course of their various business activities, the underwriters and their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (which may include bank loans and/or credit default swaps) for their own account and for the accounts of their customers and may at any time hold long and short positions in such securities and instruments. Such investments and securities activities may involve securities and/or instruments of ours or our affiliates. The underwriters and their affiliates may also make investment recommendations and/or publish or express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933, as amended, or to contribute to payments the underwriters may be required to make because of any of those liabilities.

Notice to Prospective Investors in the European Economic Area

In relation to each member state of the European Economic Area that has implemented the Prospectus Directive (each, a relevant member state), with effect from and including the date on which the Prospectus Directive is implemented in that relevant member state (the relevant implementation date), an offer of shares described in this prospectus may not be made to the public in that relevant member state other than:

to any legal entity which is a qualified investor as defined in the Prospectus Directive;

to fewer than 100 or, if the relevant member state has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the relevant Dealer or Dealers nominated by us for any such offer; or

in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of shares shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive.

For purposes of this provision, the expression an offer of securities to the public in any relevant member state means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe for the shares, as the expression may be varied in that member state by any measure implementing the Prospectus Directive in that member state, and the expression Prospectus Directive means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the relevant member state) and includes any relevant implementing measure in the relevant member state. The expression 2010 PD Amending Directive means Directive 2010/73/EU.

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The sellers of the shares have not authorized and do not authorize the making of any offer of shares through any financial intermediary on their behalf, other than offers made by the underwriters with a view to the final placement of the shares as contemplated in this prospectus. Accordingly, no purchaser of the shares, other than the underwriters, is authorized to make any further offer of the shares on behalf of the sellers or the underwriters.

Notice to Prospective Investors in the United Kingdom

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, or the Order, or (ii) high net worth entities, and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order, each such person being referred to as a relevant person . This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a relevant person should not act or rely on this document or any of its contents.

Notice to Prospective Investors in France

Neither this prospectus nor any other offering material relating to the shares described in this prospectus has been submitted to the clearance procedures of the *Autorité des Marchés Financiers* or of the competent authority of another member state of the European Economic Area and notified to the *Autorité des Marchés Financiers.* The shares have not been offered or sold and will not be offered or sold, directly or indirectly, to the public in France. Neither this prospectus nor any other offering material relating to the shares has been or will be:

- released, issued, distributed or caused to be released, issued or distributed to the public in France; or
- used in connection with any offer for subscription or sale of the shares to the public in France.

Such offers, sales and distributions will be made in France only:

- to qualified investors (*investisseurs qualifiés*) and/or to a restricted circle of investors (*cercle restreint d investisseurs*), in each case investing for their own account, all as defined in, and in accordance with articles L.411-2, D.411-1, D.411-2, D.734-1, D.744-1, D.754-1 and D.764-1 of the French *Code monétaire et financier*;
- to investment services providers authorized to engage in portfolio management on behalf of third parties; or
- in a transaction that, in accordance with article L.411-2-II-1°-or-2°-or 3° of the French *Code monétaire et financier* and article 211-2 of the General Regulations (*Règlement Général*) of the *Autorité des Marchés Financiers*, does not constitute a public offer (*appel public à l épargne*).

The shares may be resold directly or indirectly, only in compliance with articles L.411-1, L.411-2, L.412-1 and L.621-8 through L.621-8-3 of the French *Code monétaire et financier*.

Notice to Prospective Investors in Hong Kong

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong), or (ii) to professional investors within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a prospectus within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong) and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or

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elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to professional investors within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Notice to Prospective Investors in Japan

The shares offered in this prospectus have not been and will not be registered under the Financial Instruments and Exchange Law of Japan. The shares have not been offered or sold and will not be offered or sold, directly or indirectly, in Japan or to or for the account of any resident of Japan (including any corporation or other entity organized under the laws of Japan), except (i) pursuant to an exemption from the registration requirements of the Financial Instruments and Exchange Law and (ii) in compliance with any other applicable requirements of Japanese law.

Notice to Prospective Investors in Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to compliance with conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

to an institutional investor (for corporations, under Section 274 of the SFA) or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and units of shares and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions specified in Section 275 of the SFA;

where no consideration is or will be given for the transfer; or

where the transfer is by operation of law.

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Notice to Prospective Investors in Australia

No prospectus or other disclosure document (as defined in the Corporations Act 2001 (Cth) of Australia, or the Corporations Act) in relation to the common shares has been or will be lodged with the Australian Securities & Investments Commission, or ASIC. This document has not been lodged with ASIC and is only directed to certain categories of exempt persons. Accordingly, if you receive this document in Australia:

(a) you confirm and warrant that you are either:

(i) a sophisticated investor under section 708(8)(a) or (b) of the Corporations Act;

(ii) a sophisticated investor under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant s certificate to us which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made;

(iii) a person associated with the company under section 708(12) of the Corporations Act; or

(iv) a professional investor within the meaning of section 708(11)(a) or (b) of the Corporations Act, and to the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor, associated person or professional investor under the Corporations Act any offer made to you under this document is void and incapable of acceptance; and

(b) you warrant and agree that you will not offer any of the shares for resale in Australia within 12 months of that common stock being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

Notice to Prospective Investors in Chile

The shares are not registered in the Securities Registry (Registro de Valores) or subject to the control of the Chilean Securities and Exchange Commission (Superintendencia de Valores y Seguros de Chile). This prospectus and other offering materials relating to the offer of the shares do not constitute a public offer of, or an invitation to subscribe for or purchase, the shares in the Republic of Chile, other than to individually identified purchasers pursuant to a private offering within the meaning of Article 4 of the Chilean Securities Market Act (Ley de Mercado de Valores) (an offer that is not addressed to the public at large or to a certain sector or specific group of the public).

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LEGAL MATTERS

The validity of the shares of common stock being offered by this prospectus will be passed upon for us by Cooley LLP, San Diego, California. Latham & Watkins LLP, San Diego, California, is counsel for the underwriters in connection with this offering.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our consolidated financial statements at December 31, 2011 and 2012, and for each of the two years in the period ended December 31, 2012, as set forth in their report (which contains an explanatory paragraph describing conditions that raise substantial doubt about the Company s ability to continue as a going concern as described in Note 1 to the audited consolidated financial statements). We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP s report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes a part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to us and the common stock offered by this prospectus, you should refer to the registration statement and the exhibits filed as part of that document. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC s website at http://www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facilities at 100 F Street, N.E., Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities. You may also request a copy of these filings, at no cost, by writing or telephoning us at: 11080 Roselle St., San Diego, California 92121 or (858) 334-2100.

Upon the closing of this offering, we will be subject to the information and periodic reporting requirements of the Securities Exchange Act of 1934, as amended, and we will file periodic reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We also maintain a website at http://www.ambitbio.com, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of this prospectus.

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Ambit Biosciences Corporation

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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, 2011 and 2012, and the related consolidated statements of operations and comprehensive loss, statements of convertible preferred stock and stockholders deficit, and statements of cash flows for each of the two years in the period ended December 31, 2012. 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, 2011 and 2012, and the consolidated results of its operations and comprehensive loss and its cash flows for each of the two years in the period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As more fully described in Note 1, the Company has incurred recurring operating losses and has a working capital deficiency. These conditions raise substantial doubt about the Company s ability to continue as a going concern. Management s plans in regard to these matters are also described in Note 1. The December 31, 2012 financial statements do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that may result from the outcome of this uncertainty.

/s/ Ernst & Young LLP

San Diego, California

February 20, 2013

except for Note 11, as to which the date is March 26, 2013, and except for the retroactive effect of the 1-for-24 reverse stock split as described in Note 1, as to which the date is April 24, 2013

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Ambit Biosciences Corporation

Consolidated Balance Sheets

(in thousands, except share and per share data)

	December 31,		March 31,	Pro Forma March 31,
	2011	2012	2013	2013
			(unaudited)	
Assets				
Current assets:				
Cash and cash equivalents	$ 16,417	$ 17,481	$ 8,405	
Accounts receivable	3,510		994	
Prepaid expenses and other current assets	1,581	1,231	601	
Total current assets	21,508	18,712	10,000	
Property and equipment, net	1,312	560	888	
Deposits and other assets		717	1,473	
Restricted cash			63	
Total assets	$ 22,820	$ 19,989	12,424	
Liabilities, convertible preferred stock and stockholders deficit				
Current liabilities:				
Accounts payable and accrued expenses	$ 6,839	$ 7,290	$ 6,540	
Accrued payroll and related expenses	2,306	1,313	934	
Current portion of notes payable, net of discount	4,591	4,320	3,083	
Current portion of deferred gain on sale of kinase profiling services business	2,500			
Current portion of deferred revenue	6,379	6,362	16,819	
Warrant liabilities	4,916	10,540	14,497	$ 8,979
Total current liabilities	27,531	29,825	41,873	
Notes payable, net of current portion	4,320			
Other long-term liabilities	248			
Deferred revenue, net of current portion	20,671	14,309		
Redeemable non-controlling interest	1,322	3,323	7,482	
Commitments and contingencies				
Convertible preferred stock, $0.001 par value:				
Authorized shares 150,808,375 at December 31, 2011 and 170,990,763 at December 31, 2012 and March 31, 2013 (unaudited)				
Redeemable convertible preferred stock:				
Authorized shares 148,670,179 at December 31, 2011 and 168,852,567 at December 31, 2012 and March 31, 2013 (unaudited); issued and outstanding shares 75,746,862 at December 31, 2011 and 121,826,424 at December 31, 2012 and March 31, 2013 (unaudited); liquidation preference $140,072 at December 31,2011 and $202,475 at December 31, 2012 and March 31, 2013 (unaudited); no shares issued and outstanding, pro forma (unaudited)	132,340	157,076	159,395	
Convertible preferred stock:				
Authorized shares 2,138,196 at December 31, 2011 and 2012 and March 31, 2013 (unaudited); issued and outstanding shares 1,595,794 at December 31, 2011 and 1,590,014 at December 31, 2012 and March 31, 2013 (unaudited); liquidation preference $13,752 at December 31, 2011 and $13,702 at December 31, 2012 and March 31, 2013 (unaudited); no shares issued and outstanding, pro forma (unaudited)	13,752	13,702	13,702	
Stockholders deficit:				
Common stock, $0.001 par value:				
Authorized shares 175,000,000 at December 31, 2011 and 225,000,000 at December 31, 2012 and March 31, 2013 (unaudited); issued and outstanding shares 1,370 at December 31, 2011 and 3,990 at December 31, 2012 and March 31, 2013 (unaudited); 6,453,063 shares issued and				6

outstanding, pro forma (unaudited)				
Additional paid-in capital	32,941	38,678	35,258	221,349
Accumulated other comprehensive income (loss)	19	47	(86)	(86)
Accumulated deficit	(210,324)	(236,971)	(245,200)	(245,200)
Total stockholders deficit	(177,364)	(198,246)	(210,028)	$ (23,931)
Total liabilities, convertible preferred stock and stockholders deficit	$ 22,820	$ 19,989	$ 12,424	

See accompanying notes.

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Ambit Biosciences Corporation

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Revenues:				
Collaboration agreements	$ 23,843	$ 17,633	$ 5,233	$ 6,592
Operating expenses:				
Research and development	50,705	36,731	11,140	9,005
General and administrative	8,905	6,550	1,750	1,776
Gain on sale of kinase profiling services business	(2,108)	(2,497)	(555)	
Total operating expenses	57,502	40,784	12,335	10,781
Loss from operations	(33,659)	(23,151)	(7,102)	(4,189)
Other income (expense):				
Interest expense	(4,502)	(1,737)	(356)	(162)
Other income	1,538	29		7
Change in fair value of warrant and derivative liabilities	(795)	(2,291)	(547)	(3,957)
Total other income (expense)	(3,759)	(3,999)	(903)	(4,112)
Loss before income taxes	(37,418)	(27,150)	(8,005)	(8,301)
Provision (benefit) for income tax		(121)	1	1
Consolidated net loss	(37,418)	(27,029)	(8,006)	(8,302)
Net (income) loss attributable to redeemable non-controlling interest	(213)	382	98	73
Net loss attributable to Ambit Biosciences Corporation	(37,631)	(26,647)	(7,908)	(8,229)
Accretion to redemption value of redeemable convertible preferred stock	(2,000)	(3,161)	(440)	(2,319)
Change in fair value of redeemable non-controlling interest	4,477	(854)	(217)	(1,499)
Net loss attributable to common stockholders	$ (35,154)	$ (30,662)	$ (8,565)	$ (12,047)
Other comprehensive loss:				
Foreign currency translation	(242)	28	33	(133)
Comprehensive loss	$ (37,660)	$ (27,001)	$ (7,973)	$ (8,435)
Net loss per share attributable to common stockholders, basic and diluted	$ (25,886.60)	$ (16,591.99)	$ (6,251.82)	$ (3,019.30)
Weighted-average shares outstanding, basic and diluted	1,358	1,848	1,370	3,990
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)		$ (4.92)		$ (1.11)
		4,932,134		6,422,243

Pro forma weighted-average shares outstanding, basic and diluted (unaudited)	

See accompanying notes.

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Ambit Biosciences Corporation

Consolidated Statements of Convertible Preferred Stock and Stockholders Deficit

(in thousands, except share data)

	Redeemable Convertible Preferred Stock		Convertible Preferred Stock		Common Stock					
	Shares	Amount	Shares	Amount	Shares	Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Deficit
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 non-controlling interest									(213)	(213)
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)
Issuance of common stock for cash					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
Common stock warrant exchange		(197)					197			197
Issuance of common stock warrants in connection with Series E financing		(6,184)								
Cancellation of Series D-2 redeemable convertible preferred stock warrants							2,851			2,851
Cash paid for shares of Series E redeemable convertible preferred stock	31,906,341	22,334								
Series E redeemable convertible preferred stock issuance costs		(374)								
Issuance of Series E redeemable convertible preferred stock upon conversion of bridge loans	17,008,346	11,906								
Accretion to redemption value of redeemable convertible preferred stock		3,161					(3,161)			(3,161)
Change in fair value of redeemable non-controlling interest							(854)			(854)
Net loss attributable to redeemable non-controlling									382	382

interest									
Stock-based compensation						741			741
Foreign currency translation							28		28
Consolidated net loss								(27,029)	(27,029)
Balance at December 31, 2012	121,826,424	157,076	1,590,014	13,702	3,990	38,678	47	(236,971)	(198,246)
Accretion to redemption value of redeemable convertible preferred stock (unaudited)		2,319				(2,319)			(2,319)
Change in fair value of redeemable non-controlling interest (unaudited)						(1,499)			(1,499)
Net loss attributable to redeemable non-controlling interest (unaudited)								73	73
Stock-based compensation (unaudited)						398			398
Foreign currency translation (unaudited)							(133)		(133)
Consolidated net loss (unaudited)								(8,302)	(8,302)
Balance at March 31, 2013 (unaudited)	121,826,424	$ 159,395	1,590,014	$ 13,702	3,990	$ $ 35,258	$ (86)	$ (245,200)	$ (210,028)

See accompanying notes.

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Ambit Biosciences Corporation

Consolidated Statements of Cash Flows

(in thousands)

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Operating activities				
Consolidated net loss	$ (37,418)	$ (27,029)	$ (8,006)	$ (8,302)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	1,069	903	328	114
Change in fair value of warrant and derivative liabilities	795	2,291	547	3,957
Noncash interest expense	3,140	803	97	52
Bad debt expense	2			
Stock-based compensation	1,387	741	207	398
Loss (gain) on disposal of property and equipment	27	(197)	(2)	(6)
Deferred rent	(256)	(303)	(130)	
Deferred revenue	(6,362)	(6,379)	(1,586)	(3,852)
Gain on sale of kinase profiling services business	(2,108)	(2,497)	(555)	
Changes in operating assets and liabilities:				
Accounts receivable	643	3,510	3,510	(994)
Prepaid expenses and other current assets	159	(34)	(203)	630
Accounts payable and accrued expenses	(1,156)	413	1,168	(724)
Accrued payroll and related expenses	452	(994)	(957)	(379)
Net cash used in operating activities	(39,626)	(28,772)	(5,582)	(9,106)
Investing activities				
Proceeds from sale of kinase profiling services business	400			
Proceeds from sale of property and equipment			5	10
Purchase of property and equipment	(553)	46	(7)	(446)
Restricted cash				(63)
Net cash (used in) provided by investing activities	(153)	46	(2)	(499)
Financing activities				
Proceeds from exercise of stock option				
Proceeds from issuance of common stock and exercise of common stock options	79	3		
Net proceeds from issuance of redeemable convertible preferred stock	19,052	21,960		
Net proceeds from issuance of put shares	2,566	(44)		2,733
Proceeds from notes payable		13,000		
Payments on notes payable	(3,103)	(4,829)	(1,164)	(1,277)
Costs paid in connection with initial public offering		(331)		(792)
Net cash provided by (used in) financing activities	18,594	29,759	(1,164)	664
Effect of exchange rate changes on cash	(246)	31	36	(135)
Net (decrease) increase in cash and cash equivalents	(21,431)	1,064	(6,712)	(9,076)
Cash and cash equivalents at beginning of the period	37,848	16,417	16,417	17,481
Cash and cash equivalents at end of the period	$ 16,417	$ 17,481	$ 9,705	$ 8,405

Supplemental schedule of noncash investing and financing activities

Conversion of bridge notes and accrued interest into redeemable preferred stock	$ 16,138	$ 11,906	$	$
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	$	$
Supplemental disclosures of cash flow information				
Interest paid	$ 1,392	$ 982	$ 271	$ 122
Income taxes paid	$ 20	$	$	$

See accompanying notes.

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Ambit Biosciences Corporation

Notes to Consolidated Financial Statements

(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

1. Organization and Summary of Significant Accounting Policies

Organization and Business

Ambit Biosciences Corporation (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.

Liquidity

The Company has incurred losses since inception and negative cash flows from operating activities during each of 2011 and 2012 and the three months ended March 31, 2012 and 2013. As of December 31, 2012, the Company had a working capital deficit of $11.1 million and an accumulated deficit of $237.0 million. As of March 31, 2013, the Company had a working capital deficit of $31.9 million and an accumulated deficit of $245.2 million. The Company anticipates that it will continue to incur net losses into the foreseeable future as it: (i) continues the development and commercialization of its lead drug candidate quizartinib (formerly known as AC220), (ii) works to develop additional drug candidates through research and development programs, and (iii) expands its corporate infrastructure, including the costs associated with becoming a public company.

The Company plans to continue to fund its losses from operations and capital funding needs through future debt and equity financing. If the Company is not able to secure adequate additional funding, the Company may be forced to make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, and/or suspend or curtail planned programs. Any of these actions could materially harm the Company s business, results of operations, and future prospects.

The Company s recurring losses from operations, working capital deficiency and negative cash flows raise substantial doubt about its ability to continue as a going concern. The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. The Company may never become profitable, or if it does, it may not be able to sustain profitability on a recurring basis.

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). All intercompany transactions and balances are eliminated in consolidation. Ambit Europe was incorporated in England in June 2008. As of December 31, 2012, 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 reverse 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 split for all periods presented.

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Ambit Biosciences Corporation

Notes to Consolidated Financial Statements

(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

Unaudited Interim Financial Information

The accompanying interim balance sheet as of March 31, 2013 and the statements of operations and comprehensive loss and cash flows for the three months ended March 31, 2012 and 2013 and the statement of convertible preferred stock and stockholders deficit for the three months ended March 31, 2013 and the footnote disclosures are unaudited. These unaudited interim financial statements have been prepared in accordance with GAAP. In management s opinion, the unaudited interim financial statements have been prepared on the same basis as the audited financial statements and include all adjustments, which include only normal recurring adjustments, necessary for the fair presentation of the Company s financial position as of March 31, 2013 and its results of operations and comprehensive loss and its cash flows for the three months ended March 31, 2012 and 2013. The results for the three months ended March 31, 2013 are not necessarily indicative of the results expected for the full fiscal year or any other interim period.

Unaudited Pro Forma Balance Sheet Information

The unaudited pro forma balance sheet information as of March 31, 2013 assumes: (i) the conversion of all convertible preferred stock into 5,918,981 shares of common stock assuming a conversion rate of one share of common stock for each 24 shares of convertible preferred stock, except for Series D redeemable convertible preferred stock for which the conversion rate is one share of common stock for each 10.86 shares of preferred stock, (ii) the exercise and conversion of all redeemable convertible preferred stock puts into 530,092 shares of common stock, resulting in the redeemable non-controlling interest being reclassified to additional paid-in capital, and (iii) the conversion of outstanding convertible preferred stock warrants into warrants to purchase 645,598 shares of common stock resulting in the preferred stock warrant liability being reclassified to additional paid-in capital. The common stock warrant liability outstanding at March 31, 2013 will remain outstanding subsequent to the Company s initial public offering as a result of the cash settlement provisions included in the warrants. The proforma balance sheet assumes that the completion of the initial public offering contemplated by this prospectus had occurred as of March 31, 2013 and excludes shares of common stock issued in such initial public offering and any related net proceeds.

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 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 statement of operations 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 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 financial statements.

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Ambit Biosciences Corporation

Notes to Consolidated Financial Statements

(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make 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, 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.

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. 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. During 2011, 2012 and the three months ended March 31, 2013, all of the Company s revenues were from Astellas Pharma Inc.

Accounts Receivable

As of December 31, 2011 and 2012 and March 31, 2013, all accounts receivable were due from Astellas Pharma Inc. related to a collaboration agreement.

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.

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 through December 31, 2012 and March 31, 2013.

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Ambit Biosciences Corporation

Notes to Consolidated Financial Statements

(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

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. Based on the borrowing rates currently available to the Company for loans with similar terms, the Company believes that the fair value of long-term debt approximates its carrying value. The carrying amount of the warrant liabilities and redeemable non-controlling interest represent their fair values.

Warrant Liabilities

The Company has issued freestanding warrants to purchase shares of its redeemable convertible preferred stock and common stock. The redeemable convertible preferred stock warrants are exercisable for shares of Series C, Series D and Series D-2 redeemable convertible preferred stock and are classified as liabilities in the accompanying consolidated balance sheets, as the terms for redemption of the underlying security are 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 are 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. The warrant liabilities will continue to be re-measured to fair value until such time as the warrants are no longer outstanding or the underlying securities are no longer redeemable outside the control of the Company.

Deferred Rent

Rent expense, including the value of tenant improvement allowances received, is recorded on a straight-line basis over the term of the lease. The difference between rent expense and amounts paid under the lease agreements is recorded in current and other long-term liabilities in the accompanying consolidated balance sheets.

Revenues

The Company generates 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, 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

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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 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 accounted for in accordance with ASC 605-28, *Revenue Recognition Milestone Method*. 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 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 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 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.

Other

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

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 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. The adoption of this standard did not have a material impact on the Company s financial statements. The Company has not entered into nor materially modified any agreements since December 31, 2010.

Pursuant to the new standard, each required deliverable 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-

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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 collaborative agreements:

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
	(in thousands)			
Upfront licensing fees	$ 6,362	$ 6,379	$ 1,586	$ 3,852
Collaborative research activities	17,481	11,254	3,647	2,740
Total revenue from collaborative agreements	$ 23,843	$ 17,633	$ 5,233	$ 6,592

Deferred revenue

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

Research and Development Expenses

Research and development costs are expensed as incurred. Prepaid clinical expenses and advance payments for goods or 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 $980,000, $861,000 and $296,000 as of December 31, 2011 and 2012 and March 31, 2013, respectively.

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 is defined as 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 deficit for all periods presented.

Stock-Based Compensation

Stock-based compensation expense represents the cost of the grant date fair value of employee stock option grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line

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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 stock options granted (other than through a repricing) during the years ended December 31, 2011 and 2012 and the three months ended March 31, 2012 were $323.85 per share, $3.71 per share and $41.87 per share, respectively, using the Black-Scholes option pricing model with the following weighted average assumptions (annualized percentages):

	Years Ended December 31,		Three Months Ended March 31,
	2011	2012	2012
Risk-free interest rate	1.2%	0.9%	1.3%
Expected dividend yield			
Expected volatility	63.1%	67.4%	64.8%
Expected term (in years)	6.1	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 share prices are publicly available covering a timeframe similar to that of the expected term. The expected term of the options 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, 2011 and 2012 and the three months ended March 31, 2012 and 2013 is based on awards ultimately expected to vest, it should be reduced for estimated forfeitures. The Compensation-Stock Compensation Topic of the FASB Accounting Standards Codification (ASC) requires forfeitures to be estimated at the time of grant 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.

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The Company records uncertain tax positions in accordance with ASC 740 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.

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 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.

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):

	As of December 31,		As of March 31,	
	2011	2012	2012	2013
Convertible preferred stock outstanding	4,014,981	5,918,981	4,014,981	5,918,981
Redeemable non-controlling interest	273,264	366,899	273,264	530,092
Warrants for convertible preferred stock	1,208,677	645,598	1,208,677	645,598
Warrants for common stock	1,057	1,155,322	1,057	1,155,322
Common stock options	2,836	1,220,138	5,483	1,214,212
	5,500,815	9,306,938	5,503,462	9,464,205

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Unaudited Pro Forma Net Loss Per Share Attributable to Common Stockholders

The following table presents the computation of pro forma basic and diluted net loss per share attributable to common stockholders:

	Year Ended December 31, 2012	Three Months Ended March 31, 2013
	(unaudited, in thousands, except share and per share data)	
Numerator		
Net loss attributable to common stockholders	$ (30,662)	$ (12,047)
Add:		
Change in fair value of preferred stock warrant liabilities	2,293	1,160
Net loss attributable to redeemable non-controlling interest	(382)	(73)
Accretion of redeemable convertible preferred stock	3,161	2,319
Change in fair value of redeemable non-controlling interest	854	1,499
Interest on convertible notes payable	479	
	$ (24,257)	$ (7,142)
Denominator:		
Weighted-average common shares outstanding, basic and diluted	1,848	3,990
Add:		
Pro forma adjustments to reflect assumed weighted-average effect of conversion of preferred stock	4,233,943	5,918,981
Pro forma adjustments to reflect assumed weighted-average effect of conversion of convertible preferred stock issued upon conversion of debt	423,079	
Pro forma adjustments to reflect assumed weighted-average effect of exercise of put right	273,264	499,272
	4,932,134	6,422,243
Pro forma basic and diluted net loss per share attributable to common stockholders	$ (4.92)	$ (1.11)

Segments

The Company operates in a single 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.

Recently Issued Accounting Standards

Fair Value Measurement In April 2011, the FASB issued new guidance to achieve common fair value measurement and disclosure requirements between GAAP and International Financial Reporting Standards. This

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new guidance amends current fair value measurement and disclosure guidance to include increased transparency around valuation inputs and investment categorization. This new guidance is effective for fiscal years and interim periods beginning after December 15, 2011. The adoption of the new guidance did not have a material impact on the Company s consolidated financial position or results of operations.

2. Ambit Canada

Ambit Canada was incorporated on December 29, 2004. 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 has 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 since its inception through March 31, 2013.

Ambit Canada had the following shares outstanding as of March 31, 2013, December 31, 2012 and December 31, 2011:

	Shares Held by the Company	Shares Held by GrowthWorks	Shares Held by Canadian Investor	March 31, 2013
Class A voting	100	100		200
Class C non-voting	11,628,107	11,628,107		23,256,214
Series D-1 non-voting	612,649	612,649		1,225,298
Series D-2 non-voting		3,666,169		3,666,169
Class E non-voting		6,163,916		6,163,916
Class F voting (formerly Class E voting)	8		8	16
	12,240,864	22,070,941	8	34,311,813

	Shares Held by the Company	Shares Held by GrowthWorks	Shares Held by Canadian Investor	December 31, 2012
Class A voting	100	100		200
Class C non-voting	11,628,107	11,628,107		23,256,214
Series D-1 non-voting	612,649	612,649		1,225,298
Series D-2 non-voting		3,666,169		3,666,169
Class E non-voting		2,247,223		2,247,223
Class F voting (formerly Class E voting)	8		8	16
	12,240,864	18,154,248	8	30,395,120

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	Shares Held by the Company	Shares Held by GrowthWorks	Shares Held by Canadian Investor	December 31, 2011
Class A voting	100	100		200
Class C non-voting	11,628,107	11,628,107		23,256,214
Series D-1 non-voting	612,649	612,649		1,225,298
Series D-2 non-voting		3,666,169		3,666,169
Class F voting (formerly Class E voting)	8		8	16
	12,240,864	15,907,025	8	28,147,897

The Class C, Series D-1, Series D-2 and Class E shares held by GrowthWorks are subject to put options whereby GrowthWorks can exchange its non-voting shares in Ambit Canada for either: (i) cash or (ii) shares of the Company s redeemable convertible preferred stock. The election to settle the put options in cash or shares is at the sole discretion of the Company.

In May 2011, GrowthWorks received 3,666,169 shares of Ambit Canada s Series D-2 non-voting shares for consideration of $2.6 million. In addition, the Company issued GrowthWorks a warrant for the purchase of 3,101,030 shares of the Company s Series D-2 redeemable convertible preferred stock. See Note 7.

In October 2012, GrowthWorks received 2,247,223 shares of Ambit Canada s Class E non-voting shares upon the conversion of a bridge loan and related accrued interest. See Note 7.

In January 2013, the Company, Ambit Canada and GrowthWorks entered into a subscription agreement, pursuant to which Ambit Canada issued and sold 3,916,693 shares of its Class E non-voting shares to GrowthWorks for aggregate consideration of $2.7 million.

The following table summarizes the number of shares of the Company s securities that would be used to satisfy the put options:

	As of December 31,		As of March 31,
	2011	2012	2013
Series C-2 redeemable convertible preferred stock	1,538,461	1,538,461	1,538,461
Series D redeemable convertible preferred stock	612,649	612,649	612,649
Series D-2 redeemable convertible preferred stock	3,666,169	3,666,169	3,666,169
Series E redeemable convertible preferred stock		2,247,223	6,163,916
Total	5,817,279	8,064,502	11,981,195

If the put options are not exercised on or prior to the closing of a qualifying public offering, qualifying financing or sale event, the put price will be adjusted downward to equal: (i) 80.0% of the fair market value of the appropriate equity instrument in the case of a cash payment or (ii) 80.0% of the number of shares or other property in the case of payment in shares of the Company.

The Company has determined that, for all periods presented, Ambit Canada is a variable interest entity and that the Company is 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

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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 has 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 Series C, Series D, Series D-2 and Series E redeemable convertible preferred stock. At each reporting period, the Company adjusts 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 is 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 loss. The redeemable non-controlling interest will continue to be measured at fair value until the time at which no Class C, Series D, Series D-2 or Class E shares of Ambit Canada are held by GrowthWorks or any other third party, at which time the redeemable non-controlling interest will be reclassified to additional paid-in capital.

During the years ended December 31, 2011 and 2012 and the three months ended March 31, 2012 and 2013, 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 $4.5 million, $(0.9 million), $(0.2 million) and $(1.5 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 liabilities recognized as a result of consolidating Ambit Canada do not represent additional claims on the Company s general assets; rather, they represent claims against the specific assets of Ambit Canada. Conversely, assets recognized as a result of consolidating Ambit Canada do not represent additional assets that could be used to satisfy claims against the Company s general assets. The assets of Ambit Canada represent the only significant assets of the Company not located in the United States.

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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,		March 31,	
	2011	2012	2012	2013
	(in thousands)			
Cash and cash equivalents	$ 1,665	$ 2,420	$ 1,490	$ 4,943
Total assets of Ambit Canada	$ 1,665	$ 2,420	$ 1,490	$ 4,943
Accounts payable and accrued expenses	$ 87	$ 67	$ 53	$ 107
Accrued payroll and related expenses	105		105	
Total liabilities of Ambit Canada	$ 192	$ 67	$ 158	$ 107

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

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013
	(in thousands)			
Research and development expense	$ (1,026)	$ (646)	$ (173)	$ (119)
General and administrative expense	(56)			
Interest expense	(116)	(87)		6
Other income (primarily from SR&ED credit)	1,533	60		
Net income (loss) on Ambit Canada	$ 335	$ (673)	$ (173)	$ (113)

Income (loss) of Ambit Canada was allocated to the redeemable non-controlling interest based on the relative ownership of Ambit Canada. As of December 31, 2011 and 2012 and March 31, 2013, the redeemable non-controlling interest held 57%, 60%, and 64%, respectively, 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. The refundable investment tax credit is 35% of the first $3 million of qualified expenditures for SR&ED carried out in Canada, and 20% on any excess amount.

Consolidation of Ambit Canada s cash flows included the following:

	Years Ended December 31,		Three Months Ended March 31,	
	2011	2012	2012	2013

		(in thousands)		
Cash provided by (used in) operating activities	$ 394	$ (746)	$ (211)	$ (71)
Cash (used in) provided by financing activities	(2,179)	1,473		2,733
Effect of exchange rate on cash	(246)	31	36	(135)
(Decrease) increase in cash and cash equivalents of Ambit Canada	$ (2,031)	$ 758	$ (175)	$ 2,527

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3. Balance Sheet Details

Property and Equipment, net

	December 31,		March 31,
	2011	2012	2013
		(in thousands)	
Scientific Equipment	$ 3,879	$ 2,951	$ 2,864
Computer hardware and software	1,402	1,416	1,274
Furniture and fixtures	170	170	510
Leasehold improvements	1,284	1,284	23
Construction in progress		141	
	6,735	5,962	4,671
Accumulated depreciation	(5,423)	(5,402)	(3,783)
Property and equipment, net	$ 1,312	$ 560	$ 888

Accounts Payable and Accrued Expenses

	December 31,		March 31,
	2011	2012	2013
		(in thousands)	
Accounts payable	$ 4,293	$ 4,320	$ 2,608
Accrued clinical trials	1,038	996	2,174
Accrued expenses	1,050	1,609	1,380
Current portion of deferred rent	303		
Other	155	365	378
Accounts payable and accrued expenses	$ 6,839	$ 7,290	$ 6,540

4. Fair Value Measurements

The following tables and disclosure present information about the Company s financial assets and financial liabilities measured at fair value on a recurring basis as of December 31, 2011 and 2012 and March 31, 2013, and indicates the fair value hierarchy of the valuation techniques utilized by the Company to determine such fair value. As a basis for categorizing inputs, the Company uses a three-tier fair value hierarchy, which prioritizes the inputs used to measure fair value from market based assumptions to entity specific assumptions:

Level 1: Observable inputs such as quoted prices in active markets;

Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and

Level 3: Unobservable inputs in which there is little or no market data, which requires the entity to develop its own assumptions.

As of December 31, 2011 and 2012 and March 31, 2013, cash and cash equivalents are measured at face value which approximates fair value and are classified within the Level 1 designation discussed above. 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 and the redeemable

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non-controlling interest. None of the Company s non-financial assets and liabilities are recorded at fair value on a non-recurring basis. No transfers between levels have occurred during the periods presented.

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, 2011 and 2012 and March 31, 2013:

	December 31,		March 31,
	2011	2012	2013
Risk-free interest rate	1.9%	1.6%	1.8%
Expected dividend yield	0.0%	0.0%	0.0%
Expected volatility	63.8%	63.1%	62.7%
Expected term in years	9.2	9.2	8.9

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, 2011 and 2012 and March 31, 2013:

	December 31,		March 31,
	2011	2012	2013
Series C redeemable convertible preferred stock	$ 0.10	$ 0.31	$ 0.39
Series D redeemable convertible preferred stock	0.83	0.70	0.85
Series D-2 redeemable convertible preferred stock	0.18	0.31	0.39
Series E redeemable convertible preferred stock		0.57	0.80
Common stock	144.00	6.00	8.64

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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:

	Warrant Liabilities	Redeemable Non-Controlling Interest	Derivative Liability-Conversion Feature
		(in thousands)	
Balance at December 31, 2010	$ 391	$ 3,020	$ 1,624
Issuance of Series D-2 financing warrants	2,106		
Issuance of shares of redeemable non-controlling interest		2,566	
Change in fair value	2,419	(4,477)	(1,624)
Net income attributable to redeemable non-controlling interest		213	
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	2,291	854	
Net loss attributable to redeemable non-controlling interest		(382)	
Balance at December 31, 2012	10,540	3,323	
Issuance of shares of redeemable non-controlling interest		2,733	
Change in fair value	3,957	1,499	
Net loss attributable to redeemable non-controlling interest		(73)	
Balance at March 31, 2013	$ 14,497	$ 7,482	$

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5. Warrant Liabilities

The Company s outstanding warrant liabilities consisted of the following:

			March 31, 2013		
Issue Date	**Expiration Date**	**Series**	**Exercise Price per Share**	**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 2015	Series C	4.30	2,410	
September 2007	September 2017	Series C	4.30	93,023	3
August 2008	August 2016	Series D	5.06	2,369	
March 2010	March 2020	Series D-2	0.70	2,057,142	428
May 2011	May 2021	Series D-2	0.001	13,070,398	5,086
				15,491,926	5,518
Common warrants:					
October 2012	October 2022	Common	0.24	1,058,221	8,951
November 2012	October 2022	Common	0.24	3,324	28
				1,061,545	8,979
					$ 14,497

			December 31, 2012		
Issue Date	**Expiration Date**	**Series**	**Exercise Price per Share**	**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 2015	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
Total					$ 10,540

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Issue Date	Expiration Date	Series	December 31, 2011		
			Exercise Price per Share	Shares Issuable upon Exercise (in thousands except share and per share data)	Fair Value
October 2005	October 2015	Series C	$ 4.30	232,558	$
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 2015	Series C	4.30	2,410	
September 2007	September 2017	Series C	4.30	93,023	
August 2008	August 2016	Series D	5.06	2,369	
March 2010	March 2020	Series D-2	0.70	2,057,142	153
May 2011	May 2021	Series D-2	0.001	26,583,858	4,763
Total				29,005,386	$ 4,916

6. Debt, Commitments and Contingencies

Debt

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

	December 31,		March 31,
	2011	2012	2013
		(in thousands)	
Equipment notes payable	$ 93	$	$
Venture loans	9,149	4,412	3,135
Total notes payable	9,242	4,412	3,135
Discount on notes payable	(331)	(92)	(52)
Total notes payable, net of debt discount	8,911	4,320	3,083
Current portion of notes payable	(4,829)	(4,412)	(3,135)
Current portion of debt discount	238	92	52
Current portion of notes payable, net of debt discount	(4,591)	(4,320)	(3,083)

Notes payable, net of current portion	$ 4,320	$	$

Equipment Notes Payable

The Company had outstanding promissory notes under two master security agreements secured by the related equipment; however, no funds are available for future borrowings. In connection with these notes, the Company issued the lenders various warrants to purchase shares of Company s convertible preferred stock and redeemable convertible preferred stock. The initial fair value of the warrants, aggregating approximately $0.1 million, was recorded as a debt discount and was amortized to interest expense over the term of the note on

Ambit Biosciences Corporation

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(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

the effective interest method. As of December 31, 2011, the remaining promissory note outstanding required monthly principal and interest payments and had an interest rate of 11.25%. This note was fully paid in August 2012.

Venture Loans

On March 31, 2010, the Company 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 (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 due on October 1, 2013 includes additional interest of 3.0% of the initial loan amount, or $360,000, which is being accreted over the life of the notes using the effective interest method and is included in interest expense. 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 are secured by a first priority security interest in all assets, excluding intellectual property, for which the Company has 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. The warrants are exercisable for the purchase of an aggregate of 2,057,142 shares of the Company s Series D-2 redeemable convertible preferred stock at an exercise price of $0.70 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.

Rent expense was $2.4 million, $1.2 million, $416,000 and $156,000 in the years ended December 31, 2011 and 2012 and the three months ended March 31, 2012 and 2013, respectively. The Company received sublease income of $0.1 million for the year ended December 31, 2011 and no material amounts were received in 2012 or in the three months ended March 31, 2013.

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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.

Commitments

Payment schedules for commitments and contractual obligations as of December 31, 2012 are as follows:

	Notes Payable	Operating Lease (in thousands)	Total
Period ending December 31,			
2013	$ 5,024	$ 615	$ 5,639
2014		739	739
2015		758	758
2016		659	659
2017		680	680
Thereafter		525	525
Total minimum payments	5,024	$ 3,976	$ 9,000
Less interest	(252)		
Less debt discount and final payment	(452)		
Less current portion of notes payable	(4,320)		
Notes payable, net of current portion	$		

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, 2012, 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.

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7. Redeemable Convertible Preferred Stock, Convertible Preferred Stock and Stockholders Deficit

The authorized, issued and outstanding shares of stock by series are as follows as of December 31, 2012 and March 31, 2013:

	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

The authorized, issued and outstanding shares of stock by series are as follows as of December 31, 2011:

	Shares Authorized	Shares Outstanding	Liquidation Preference Per Share	Liquidation Preference (in thousands)
Redeemable convertible preferred stock:				
Series C	7,076,718	5,139,734	$ 4.30	$ 22,101
Series C-2	1,538,461		3.25	
Series D-0	16,335,000		5.06	
Series D	17,000,000	15,721,545	5.06	79,551
Series D-2	101,000,000	54,885,583	0.70	38,420
Series D-3	5,720,000		1.40	
Total	148,670,179	75,746,862		$ 140,072
Convertible preferred stock:				
Series A	162,519	46,666	$ 7.50	$ 351
Series B	1,975,677	1,549,128	8.65	13,401
Total	2,138,196	1,595,794		$ 13,752

Description of Securities

Conversion

With the exception of the Series D redeemable convertible preferred stock, all shares of preferred stock are convertible at the option of the holder into one share of common stock for each 24 shares of preferred stock, subject to adjustment. Series D preferred stock is convertible into one share of common stock for each 10.86

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shares of preferred stock. Each share of preferred stock is automatically convertible into common stock, at its then effective conversion price, (i) upon the election of the holders of at least 60% of the outstanding Series E preferred stock or (ii) upon the closing of a firmly underwritten public offering in which the pre-money valuation is at least $125 million and results in net cash proceeds of at least $50 million. Shares of preferred stock may also become automatically convertible into common stock upon a stockholder s failure to purchase their specified amount in the Series E financing.

Dividends

Each holder of preferred stock is entitled to non-cumulative dividends at an annual rate of 8.0% of the original issue price when and if declared by the Board of Directors. Dividends are paid with the following preference: (i) Series E, (ii) Series D and Series D-2, (iii) Series C and Series C-2, (iv) Series A and Series B and, finally, (v) common stock. If dividends are paid to the holders of common stock the holders of preferred stock will participate as if they had converted to common stock.

As of March 31, 2013, the Board of Directors of the Company has not declared any dividends. In connection with the covenants provided in the Venture Loan, the Company has agreed not to pay any dividends without the consent of the lenders.

Liquidation Preferences

Liquidation amounts are paid with the same preference as the dividends above, with the exception that Series B preferred stock is paid in preference to Series A preferred stock. Once all series of preferred stock have been paid the liquidation preference, plus declared but unpaid dividends, all remaining assets of the Company would be distributed to holders of capital stock as if each share had been converted to common stock.

Voting

The holder of each share of preferred stock is entitled to one vote for each share of common stock into which it would convert.

Redemption Requirements

The Series E redeemable convertible preferred stock is redeemable if, after five years from October 2012, the holders of at least an aggregate of 60% of Series E shares elect to cause the Company to redeem the stock. The redemptions shall occur in one cash installment at the greater of (i) the fair market value of the Series E redeemable convertible preferred stock and (ii) two times the original issue price of $0.70 per share, subject to adjustment, plus any declared but unpaid dividends, with the first redemption occurring within 60 days of written notice.

The Series D and Series D-2 redeemable convertible preferred stock are redeemable if (i) all of the outstanding shares Series E redeemable convertible stock have been redeemed and (ii) the holders of at least an aggregate of 67% of Series D and Series D-2 shares elect to cause the Company to redeem the stock. The redemptions shall occur over 12 equal quarterly installments at an initial price of $5.06 per share for Series D shares and at an initial price of $0.70 per share for the Series D-2 shares, subject to adjustment, plus any declared but unpaid dividends, with the first redemption occurring within 60 days of written notice.

The Series C and Series C-2 redeemable convertible preferred stock become subject to redemption if: (i) all of the outstanding shares of Series D and Series D-2 redeemable convertible preferred stock have been redeemed

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and (ii) the holders of at least 67% of the Series C redeemable convertible preferred stock and the Series C-2 redeemable convertible preferred stock shares elect to cause the Company to redeem the stock. The redemptions shall occur over 12 equal quarterly installments at an initial price of $4.30 per share for Series C shares and at an initial price of $3.25 per share for the Series C-2 shares, subject to adjustment, plus any declared but unpaid dividends, with the first redemption occurring within 60 days of written notice.

The Company is accreting the carrying amounts of the redeemable convertible preferred stock up to the redemption amount on a straight-line basis. The aggregate amount of redemption requirements as of December 31, 2012 for all series of redeemable convertible preferred stock outstanding, excluding warrants and put shares, that are redeemable assuming exercise of redemption rights at the earliest possible date, is as follows:

	Series C	Series D and D-2	Series E	Total
		(in thousands)		
Years ending December 31,				
2013	$	$	$	$
2014				
2015				
2016				
2017		9,596	68,481	78,077
Thereafter	18,837	105,561		124,398
	$ 18,837	$ 115,157	$ 68,481	$ 202,475

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.

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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.

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 is scheduled to close in three tranches. The financing agreement contains provisions whereby certain existing investors that do 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

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$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 $131,824 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.

The second tranche closing is scheduled to take place at the earlier of (i) a date and time determined by the Chief Financial Officer of the Company with notice to the investors that the Company s cash balance is less than $5.0 million and (ii) the closing date of the Company s initial public offering. If the second tranche closing occurs concurrent with the closing of the Company s initial public offering, the Company will issue shares of its common stock at a per share price equal to the lower of the public offering price or the effective conversion price of one share of its Series E redeemable convertible preferred stock immediately prior to the closing for aggregate gross proceeds of $8.9 million. If the second tranche closing occurs prior to the closing of the Company s initial public offering, the Company will issue 12,762,538 shares of its Series E redeemable convertible preferred stock for aggregate gross proceeds of $8.9 million.

The third tranche closing is scheduled to take place concurrently with the Company s initial public offering. In the third tranche closing, the Company will issue shares of its common stock at a per share price equal to the public offering price for aggregate gross proceeds of $13,400,684.

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Common Warrants

The following table summarizes the warrants outstanding for purchase of common stock as of December 31, 2012 and March 31, 2013 (excluding warrants that require liability accounting):

Shares Issuable upon Exercise	Exercise Price	Expiration Date
70,704	$ 21.84	June 2019
2,266	21.84	July 2019
20,690	36.96	September 2020
78	2,184.00	July 2019
39	3,696.00	September 2020
93,777		

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, 2011 and 2012 and March 31, 2013:

	December 31,		March 31,
	2011	2012	2013
Redeemable convertible preferred stock and convertible preferred stock	4,014,981	5,918,981	5,918,981
Redeemable non-controlling interest	273,264	366,899	530,092
Warrants for redeemable convertible preferred stock and convertible preferred stock	1,208,677	645,598	645,598
Warrants for common stock	1,057	1,155,322	1,155,322
Common stock option grants issued and outstanding	2,836	1,220,138	1,214,212
Common shares available for grant under the stock option plan	3,824	191	6,117
Total common shares reserved for future issuance	5,504,639	9,307,129	9,470,322

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.

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As of December 31, 2012 and March 31, 2013, 191 and 6,117 shares, respectively, remained available for future grant.

In August 2011, the Board of Directors authorized the repricing of the exercise price of 1,291 options previously granted to employees, consultants and directors. These cancelled and repriced options are included as options granted and cancelled during 2011 in the table below. The Company analysed 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 a strike price above the fair market value of the Company s common stock.

The following table summarizes stock option activity under the Plan:

	Number of Options	Weighted-Average Exercise Price
Options outstanding, December 31, 2011	2,836	$ 974.52
Granted	1,218,134	$ 7.65
Cancelled	(832)	$ 1,098.70
Options outstanding, December 31, 2012	1,220,138	$ 9.15
Cancelled	(5,926)	$ 91.97
Options outstanding, March 31, 2013	1,214,212	$ 8.75

The following table summarizes information about the Company s stock option plan as of March 31, 2013 and December 31, 2012:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
March 31, 2013:				
Options vested and expected to vest	961,535	$ 9.25	9.6	$ 2,526
Options exercisable	112,381	$ 19.84	8.8	$ 291
December 31, 2012:				
Options vested and expected to vest	890,681	$ 9.92	9.8	$
Options exercisable	37,147	$ 52.08	7.4	$

The following table summarizes information about stock options:

	Years Ended December 31,	
	2011	2012
	(in thousands, except contractual term)	

Weighted-average remaining contractual term (years) of options outstanding	6.3	9.9
Aggregate intrinsic value of options outstanding	$	$
Intrinsic value of options exercised	$ 34	$
Cash received upon exercise of stock options	$ 79	$

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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,		Three Months Ended March 31,	
	2011	2012	2012	2013
	(in thousands)			
Research and development	$ 224	$ 129	$ 39	$ 111
General and administrative	1,163	612	168	287
	$ 1,387	$ 741	$ 207	$ 398

As of March 31, 2013, total unrecognized stock-based compensation costs related to non-vested stock options was approximately $3.6 million and the weighted-average period over which it is expected to be recognized is approximately 3.6 years. As of December 31, 2012, total unrecognized stock-based compensation costs related to non-vested stock options was approximately $3.8 million and the weighted-average period over which it is expected to be recognized is approximately 3.8 years.

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.

The parties share oversight of the research and development programs in the United States and European Union, including regulatory filings, through a joint committee to which the Company and Astellas contribute equal representation. In the event of any conflict regarding a joint committee decision, Astellas has final decision-making authority, subject to the Company s right to opt out of funding certain additional development costs associated with Astellas decision. Under the agreement, both parties are obligated to use commercially reasonable efforts to perform the tasks and activities assigned to such party under each research and development plan. The Company equally shares with Astellas the agreed-upon research and development costs in the United States and the European Union for quizartinib and the follow-on compounds, including, among others, costs related to manufacturing, labor, materials and services provided by third parties. Astellas is solely responsible for research and development costs associated with the products covered by the agreement outside the United States and the European Union and 100% of worldwide commercialization costs. The Company has operational responsibility for the manufacture and supply of all quantities of quizartinib to Astellas for a limited period of time to ensure the successful transfer of manufacturing technology to Astellas. Astellas has the sole right and option, at its own expense, to make regulatory filings associated with the products covered by the agreement outside the United States and to determine the contents of such filings.

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Upon commercialization, Astellas is responsible for all worldwide commercialization activities while the Company is entitled to receive from Astellas tiered royalty payments that would range from a low- to mid-teens percentage of net sales of up to $500.0 million, together with additional annual sales-based milestone payments. However, the Company has the option, exercisable during certain periods, to co-promote and share equally in the profit and losses from commercialization activities within the United States rather than receiving royalties on sales in the United States. To date, the Company has not exercised the co-promotion option. Astellas royalty obligations are payable on a product-by-product, country-by-country basis beginning on the date of the first commercial sale of a licensed product in a country and ending on the later of 10 years after the date of such first commercial sale in that country (or the European Union) or the expiration date of the last relevant patent or regulatory exclusivity period.

The Company s agreement with Astellas provides that, in the event the Company experiences a change of control, for three months following the transaction, Astellas may terminate any co-promotion agreement that the Company has entered into with Astellas in connection with a prior exercise of the Company s co-promotion option. Following such termination, the Company would continue to forego royalties on sales of products covered by such co-promotion agreement in exchange for a 50% share of profits and losses; however, Astellas would have full control over all commercialization activities in the United States and would be entitled to field the entire sales force and include costs incurred to build and maintain and operate its sales force in the calculation of its expenses for purposes of calculating the co-promotion payments payable to the Company. In addition, within three months after the Company experiences a change of control, Astellas would be entitled to terminate: any co-promotion option to the extent the Company has not yet exercised it; the Company s involvement in the co-development of products under the agreement; and/or the Company s role in all collaborative activities under the agreement.

The agreement expires upon the expiration of all royalty or other payment obligations under the agreement. Upon expiration of the agreement, Astellas licenses become fully paid-up, perpetual, non-exclusive licenses and neither party has further rights or obligations under the agreement, other than certain rights intended to survive expiration under the agreement. Astellas may terminate the agreement for convenience and without cause on a country-by-country, product-by-product basis upon delivery of 180 days written notice to the Company. Upon delivery of 30 days written notice to the Company, Astellas may terminate the agreement on a product-by-product basis for safety or regulatory concerns (provided the Company concurs with the basis of concern), or on a product-by-product, country-by-country basis if Astellas concludes reasonably and in good faith that continued development or commercialization will infringe upon the patent rights of a third-party or a third-party institutes or threatens suit against the Company or Astellas, claiming that development or commercialization of a licensed product infringes or misappropriates its patent rights and Astellas concludes reasonably and in good faith that there is a substantial likelihood that such suit will be successful. Either party may terminate the agreement for the other party s uncured material breach. Also, a party s dissolution, liquidation, bankruptcy or insolvency gives the other party a right to terminate. Upon termination of the agreement by Astellas for convenience or due to safety or regulatory concerns or anticipated patent infringement, or termination by the Company in the case of Astellas material breach bankruptcy or insolvency, the licenses and rights granted to Astellas under the agreement terminate on a product-by-product and country-by-country basis, as applicable.

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 is being recognized on a

Ambit Biosciences Corporation

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(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

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.

Upon the successful achievement of clinical development and regulatory milestones, the Company is eligible to receive from Astellas up to 34 milestones, totaling up to $350.0 million, of which ten milestones, totaling up to $160.0 million, are related to the AML indication. At the inception of the agreement, the Company undertook a review of the milestones specified by the agreement. It was determined that all milestones were substantive as the achievability of each milestone was not reasonably assured at the onset of the agreement. All milestones were determined to be due either to the Company s performance or upon the occurrence of a specific outcome resulting from the Company s performance. As such, all milestones are accounted for in accordance with ASC 605-28, *Revenue Recognition Milestone Method*.

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 will continue to share agreed-upon development costs equally. Subsequent to September 2, 2013, the Company will be solely responsible for development costs associated with quizartinib.

The Company recorded revenues under this agreement of $23.8 million, $17.6 million, $5.2 million and $6.6 million in the years ended December 31, 2011 and 2012 and the three months ended March 31, 2012 and 2013, respectively. Deferred revenues under this agreement were $27.1 million, $20.7 million and $16.8 million as of December 31, 2011 and 2012 and March 31, 2013, respectively.

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,

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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.

The Company recorded no revenues under this agreement in the years ended December 31, 2011 and 2012. There were no deferred revenues under this agreement as of December 31, 2011 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 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.

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(Information as of March 31, 2013 and thereafter and for the three months ended March 31, 2012 and 2013 is unaudited)

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,	
	2011	2012
	(in thousands)	
United States operations	$ (37,753)	$ (26,477)
Foreign operations	335	(673)
	$ (37,418)	$ (27,150)

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

	Years Ended December 31,	
	2011	2012
	(in thousands)	
Current:		
Federal	$	$ (122)
State and local		1
Foreign		
Total current		(121)
Total deferred		
Total provision	$	$ (121)

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

	Years Ended December 31,	
	2011	2012

Income tax benefit at federal statutory rate	(35.0)%	(35.0)%
Income tax benefit at state statutory rate	(5.8)%	(5.6)%
Research and development credits	(1.8)%	(0.7)%
Change in valuation allowance	40.6%	36.1%
Accretion	0.9%	3.4%
Other, net	1.1%	1.3%
	0.0%	(0.5)%

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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,	
	2011	2012
	(in thousands)	
Deferred tax assets:		
Net operating loss carryovers	$ 48,510	$ 62,730
Deferred revenues	11,022	8,423
Research and development credits	10,930	11,257
Intangible assets	7,300	6,621
Other	3,768	2,313
Total deferred tax assets	81,530	91,344
Deferred tax liabilities:		
Other comprehensive income	(8)	(19)
Total deferred tax liabilities	(8)	(19)
Net deferred tax asset	81,522	91,325
Valuation allowance	(81,522)	(91,325)
Net deferred tax assets	$	$

At December 31, 2011 and 2012, the Company had federal net operating loss carryforwards of approximately $117.2 million and $151.2 million, respectively. At December 31, 2011 and 2012, the Company had state net operating loss carryforwards of $106.7 million and $143.1 million, respectively. The federal and state tax loss carryforwards will begin to expire in 2022 and 2013, respectively, unless previously utilized. At December 31, 2011 and 2012, the Company also had federal research and development tax credit carryforwards of approximately $5.0 million, which will begin expiring in 2024 unless previously utilized. The American Taxpayer Relief Act of 2012 (the Act) was signed into law on January 2, 2013. The Act retroactively restored several expired business tax provisions, including the federal research and development tax credit. Management expects the Act s extension of these provisions through the end of 2013 will also result in additional deferred tax assets offset by a full valuation allowance. At December 31, 2011 and 2012, the Company also had state tax credit carryforwards of approximately $5.3 million and $5.7 million, respectively, which carry forward indefinitely. At December 31, 2011 and 2012 the Company had Canadian net operating loss carryforwards of approximately $5.4 million and $6.3 million, respectively, which begin to expire in 2026 unless previously utilized. At December 31, 2011 and 2012, the Company also had Canadian tax credits of $4.3 million and $4.4 million, respectively, which carryforward indefinitely.

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

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Ambit Biosciences Corporation

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ownership change actually occurred during 2004 or 2005 and, because a change may have occurred, the Company has reduced both its federal and state net operating loss carryforwards by approximately $5.5 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 2011 and 2012, the Company has not performed any additional analysis for IRC Sections 382 and 383 for the years ended December 31, 2011 and 2012.

The following table summarizes the changes in the Company s unrecognized tax benefits during the year ended December 31, 2012 (in thousands):

Gross unrecognized tax benefits at December 31, 2010	$ 2,026
Increase in current year position	275
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

The gross unrecognized tax benefits as of December 31, 2012 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, 2012 will change within the next twelve months. During the years ended December 31, 2011 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 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. As of December 31, 2012, there have been no purchase adjustments. As of December 31, 2011, deferred gain related to this transaction was $2.5 million.

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11. Subsequent Events

Astellas Collaboration Agreement

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 will continue to share agreed-upon development costs equally. Subsequent to September 2, 2013, the Company will be solely responsible for development costs associated with quizartinib.

Agreement to Exercise Put Right (unaudited)

In April 2013, GrowthWorks and the Company entered into a letter agreement whereunder GrowthWorks agreed to exercise the GrowthWorks put right contingent and effective upon the conversion of all the Company s outstanding shares of preferred stock into shares of the Company s common stock. Upon such exercise, GrowthWorks will receive that number of shares of the Company s common stock as is equal to the number of shares of the Company s common stock that it would have received had it exercised the GrowthWorks put right immediately prior to the conversion of all of the Company s preferred stock into shares of the Company s common stock. Following the exercise of the GrowthWorks put right, GrowthWorks shall have no further rights to acquire the Company s stock pursuant to the GrowthWorks put right and Ambit Canada will be a wholly-owned subsidiary of the Company.

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4,645,000 Shares

Common Stock

PRELIMINARY PROSPECTUS

, 2013

Citigroup

Leerink Swann

BMO Capital Markets

Baird

Through and including , 2013 (25 days after the commencement of this offering), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

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PART II

INFORMATION NOT REQUIRED IN PROSPECTUS

Item 13. Other expenses of issuance and distribution.

The following table sets forth all costs and expenses, other than underwriting discounts and commissions, paid or payable by us in connection with the sale of the common stock being registered. All amounts shown are estimates except for the SEC registration fee, the FINRA filing fee and the listing fee for the Nasdaq Global Market.

	Amount Paid or to be Paid
SEC registration fee	$ 10,929
FINRA filing fee	13,438
The Nasdaq Global Market listing fee	125,000
Blue sky qualification fees and expenses	20,000
Printing and engraving expenses	450,000
Legal fees and expenses	1,400,000
Accounting fees and expenses	700,000
Transfer agent and registrar fees and expenses	20,000
Miscellaneous expenses	110,633
Total	$ 2,850,000

Item 14. Indemnification of directors and officers.

We are incorporated under the laws of the State of Delaware. Section 145 of the Delaware General Corporation Law provides that a Delaware corporation may indemnify any persons who are, or are threatened to be made, parties to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of such corporation), by reason of the fact that such person was an officer, director, employee or agent of such corporation, or is or was serving at the request of such person as an officer, director, employee or agent of another corporation or enterprise. The indemnity may include expenses (including attorneys fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding, provided that such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the corporation s best interests and, with respect to any criminal action or proceeding, had no reasonable cause to believe that his or her conduct was illegal. A Delaware corporation may indemnify any persons who are, or are threatened to be made, a party to any threatened, pending or completed action or suit by or in the right of the corporation by reason of the fact that such person was a director, officer, employee or agent of such corporation, or is or was serving at the request of such corporation as a director, officer, employee or agent of another corporation or enterprise. The indemnity may include expenses (including attorneys fees) actually and reasonably incurred by such person in connection with the defense or settlement of such action or suit provided such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the corporation s best interests except that no indemnification is permitted without judicial approval if the officer or director is adjudged to be liable to the corporation. Where an officer or director is successful on the merits or otherwise in the defense of any action referred to above, the corporation must indemnify him or her against the expenses which such officer or director has actually and reasonably incurred. Our amended and restated certificate of incorporation and amended and restated bylaws, each of which will become effective upon the closing of this offering, provide for the indemnification of our directors and officers to the fullest extent permitted under the Delaware General Corporation Law.

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Section 102(b)(7) of the Delaware General Corporation Law permits a corporation to provide in its certificate of incorporation that a director of the corporation shall not be personally liable to the corporation or its stockholders for monetary damages for breach of fiduciary duties as a director, except for liability for any:

- transaction from which the director derives an improper personal benefit;
- act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payment of dividends or redemption of shares; or
- breach of a director s duty of loyalty to the corporation or its stockholders.

Our amended and restated certificate of incorporation and amended and restated bylaws include such a provision. Expenses incurred by any officer or director in defending any such action, suit or proceeding in advance of its final disposition shall be paid by us upon delivery to us of an undertaking, by or on behalf of such director or officer, to repay all amounts so advanced if it shall ultimately be determined that such director or officer is not entitled to be indemnified by us.

Section 174 of the Delaware General Corporation Law provides, among other things, that a director who willfully or negligently approves of an unlawful payment of dividends or an unlawful stock purchase or redemption may be held liable for such actions. A director who was either absent when the unlawful actions were approved, or dissented at the time, may avoid liability by causing his or her dissent to such actions to be entered in the books containing minutes of the meetings of the board of directors at the time such action occurred or immediately after such absent director receives notice of the unlawful acts.

As permitted by the Delaware General Corporation Law, we have entered into indemnity agreements with each of our directors and executive officers, that require us to indemnify such persons against any and all expenses (including attorneys fees), witness fees, damages, judgments, fines, settlements and other amounts incurred (including expenses of a derivative action) in connection with any action, suit or proceeding, whether actual or threatened, to which any such person may be made a party by reason of the fact that such person is or was a director, an officer or an employee of Ambit or any of its affiliated enterprises, provided that such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to our best interests and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful. The indemnification agreements also set forth certain procedures that will apply in the event of a claim for indemnification thereunder.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or preceding that may result in a claim for indemnification.

We have an insurance policy covering our officers and directors with respect to certain liabilities, including liabilities arising under the Securities Act or otherwise.

We have entered into an underwriting agreement which provides that the underwriters are obligated, under some circumstances, to indemnify our directors, officers and controlling persons against specified liabilities, including liabilities under the Securities Act.

Reference is made to the following documents filed as exhibits to this registration statement regarding relevant indemnification provisions described above and elsewhere herein:

Exhibit Document	Number
Form of Underwriting Agreement	1.1
Form of Amended and Restated Certificate of Incorporation to be effective upon the closing of this offering	3.3
Form of Amended and Restated Bylaws to be effective upon the closing of this offering	3.7

Form of Indemnity Agreement	10.1
Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Registrant and certain of its stockholders, as amended	10.18

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Item 15. ***Recent sales of unregistered securities.***

The following list sets forth information regarding all securities sold by us since January 2010.

(1) In March 2010, in connection with a venture loan with Compass Horizon Funding Company LLC and Oxford Finance Corporation, we issued a warrant to each of Horizon and Oxford to purchase an aggregate of 284,584 shares of our Series D redeemable convertible preferred stock or, at the option of the holders of the warrants, 2,057,142 shares of our Series D-2 redeemable convertible preferred stock. The exercise price of these warrants is $5.06 per share if exercised for our Series D redeemable convertible preferred stock or of $0.70 per share if exercised for our Series D-2 redeemable convertible preferred stock. Upon the closing of this offering, these warrants will be exercisable for an aggregate of 85,714 shares of our common stock at an exercise price of $16.80 per share. These warrants terminate ten years after the date issued.

(2) In September 2010, we issued secured subordinated convertible promissory notes in an aggregate amount of $13,253,367 each with a maturity date of January 31, 2012. These promissory notes converted into 23,966,205 shares of Series D-2 redeemable convertible preferred stock. In October and November 2012, an aggregate of 1,240,068 of these shares were converted into 516 shares of our common stock and the remaining shares will convert into 946,922 shares of our common stock upon completion this offering.

(3) In September 2010, Ambit Canada also issued a promissory note on substantially the same terms as the notes issued by Ambit, in the principal amount of $1,745,810 to GrowthWorks Canadian Fund Ltd, with a maturity date of January 31, 2012. This note was cancelled in connection with the conversion of the convertible promissory notes set forth in (2) above.

(4) In September 2010, in connection with our bridge financing and Ambit Canada s bridge financing, we issued warrants to purchase shares of our common stock, with an exercise price of $36.96 per share. As of November 30, 2012, certain of these warrants were exchanged or became exchangeable as described in (13) below and the remaining warrants were exercisable for an aggregate of 39 shares of our common stock at an exercise price of $3,696.00 per share. These warrants terminate 10 years after the date issued.

(5) In September 2010, in connection with Ambit Canada s issuance of the note in (3) above, we issued a warrant to purchase preferred stock to GrowthWorks Canadian Fund Ltd. This warrant was automatically exercised for 3,156,967 shares of or Series D-2 redeemable convertible preferred stock when the convertible promissory note set forth in (3) above was cancelled in connection with the conversion of the convertible promissory notes issued by Ambit in the 2010 bridge financing. These shares of our Series D-2 redeemable convertible preferred stock will convert into 131,540 shares of our common stock upon completion of this offering.

(6) In May 2011, in connection with our Series D-2 preferred stock financing, we issued and sold an aggregate of 27,762,411 shares of our Series D-2 redeemable, convertible preferred stock to our investors at a purchase price of $0.70 per share, for aggregate gross proceeds of $19.4 million. In October and November 2012, an aggregate of 523,809 of these shares were converted into 218 shares of our common stock and the remaining shares will convert into 1,134,942 shares of our common stock upon completion of this offering.

(7) In May 2011, in connection with our Series D-2 preferred stock financing, Ambit Canada issued and sold 3,666,169 shares of its Series D-2 preferred shares to GrowthWorks Canadian Fund Ltd. at a purchase price of $0.70 per share, for aggregate gross proceeds of $2.6 million. Upon completion of this offering, these shares will convert into 152,757 shares of our common stock.

(8) In May 2011, in connection with our Series D-2 preferred stock financing and the issuance of Series D-2 preferred shares by Ambit Canada, we issued warrants to purchase shares of our Series D-2 redeemable convertible preferred stock, with an initial exercise price of $0.001 per share. As of December 31, 2012, these warrants were exercisable for an aggregate of 13,070,398 shares. These

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warrants will become exercisable for an aggregate of 544,599 shares of our common stock upon the completion of this offering. These warrants terminate 10 years after the date issued.

(9) In May and July 2012, we issued secured subordinated convertible promissory notes in an aggregate amount of $11.5 million each with a maturity date of April 30, 2015. These promissory notes converted into 17,008,346 shares of Series E redeemable convertible preferred stock which will convert into 708,681 shares of our common stock upon completion this offering.

(10) In May and July 2012, Ambit Canada also issued a promissory note on substantially the same terms as the notes issued by Ambit, in the principal amount of $1.5 million to GrowthWorks Canadian Fund Ltd, with a maturity date of April 30, 2015. These promissory notes converted into 2,247,223 shares of Ambit Canada s Class E preferred stock which will convert into 93,634 shares of our common stock upon completion this offering.

(11) In October and November 2012, in connection with our Series E preferred stock financing, we issued and sold an aggregate of 1,437 shares (after the 1-for-100 reverse stock split on the common stock effected on October 26, 2012) of our common stock to our investors at a purchase price of $2.40 per share and an aggregate of 31,906,341 shares of our Series E redeemable convertible preferred stock to our investors at a purchase price of $0.70 per share, for aggregate gross proceeds of $22.3 million (not including shares issued upon conversion of indebtedness described in note (9) above). Upon completion of this offering, these shares of Series E redeemable convertible preferred stock will convert into 1,329,430 shares of our common stock.

(12) In October and November 2012, in connection with our Series E preferred stock financing, we issued warrants to purchase an aggregate of 1,061,550 shares of our common stock, with an initial exercise price of $0.24 per share. These warrants terminate 10 years after the date issued.

(13) In November 2012 and December 2012, in connection with the Series E preferred stock financing, warrants to purchase an aggregate of up to 729 shares of our common stock held by certain qualified investors were exchanged or became exchangeable for warrants to purchase an aggregate of up to 72,970 shares of our 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. Also in November 2012 and December 2012, in connection with the Series E preferred stock financing, warrants to purchase an aggregate of up to 206 shares of our common stock held by certain qualified investors were exchanged or became exchangeable for warrants to purchase an aggregate of up to 20,690 shares of our 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.

(14) In January 2013, in connection with our Series E preferred stock financing, 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. Upon completion of this offering, these shares will convert into 163,195 shares of our common stock.

(15) From January 1, 2009 to March 31, 2013, we granted stock options under our 2011 pre-IPO plan to purchase 1,213,029 shares of common stock (net of expirations and cancellations) to our employees, directors and consultants, having exercise prices ranging from $6.00 to $1,992.00 per share. Of these, no options have been exercised through March 31, 2013.

The offers, sales and issuances of the securities described in paragraphs (1) through (14) were deemed to be exempt from registration under the Securities Act in reliance on Rule 506 of Regulation D in that the issuance of securities to the accredited investors did not involve a public offering. The recipients of securities in each of these transactions acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions was an accredited investor under Rule 501 of Regulation D.

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The offers, sales and issuances of the securities described in paragraph (15) were deemed to be exempt from registration under the Securities Act in reliance on Rule 701 in that the transactions were under compensatory benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of such securities were our employees, directors or bona fide consultants and received the securities under our 2011 pre-IPO plan. Appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us.

Item 16. ***Exhibits and financial statement schedules.***

(a) Exhibits.

Exhibit Number	Description of Document
1.1#	Form of Underwriting Agreement.
3.1#	Amended and Restated Certificate of Incorporation, as currently in effect.
3.2#	Certificate of Amendment to Amended and Restated Certificate of Incorporation, as currently in effect.
3.2B	Second Certificate of Amendment to Amended and Restated Certificate of Incorporation, as currently in effect.
3.3#	Form of Amended and Restated Certificate of Incorporation to be effective upon completion of this offering.
3.4#	Bylaws, as currently in effect.
3.5#	Amendment to the Bylaws, as currently in effect.
3.6#	Second Amendment to the Bylaws, as currently in effect.
3.7#	Form of Amended and Restated Bylaws to be effective upon completion of this offering.
4.1#	Form of Common Stock Certificate.
4.2#	Form of Warrant to Purchase Common Stock issued by the Registrant to June, July and December 2009 bridge financing investors.
4.3#	Form of Warrant to Purchase Common Stock issued by the Registrant to September 2010 bridge financing investors.
4.4#	Warrant issued by the Registrant on October 5, 2005 to Oxford Finance Corporation.
4.5#	Warrant issued by the Registrant on December 22, 2005 to Oxford Finance Corporation.
4.6#	Form of Warrant issued by the Registrant to Oxford Finance Corporation pursuant to 2006 Master Security Agreement.
4.7#	Form of Warrant issued by the Registrant to Webster Bank, National Association pursuant to 2006 Master Security Agreement.
4.8#	Warrant issued by the Registrant on October 6, 2005 to Horizon Technology Funding Company II, LLC.
4.9#	Warrant issued by the Registrant on October 6, 2005 to Horizon Technology Funding Company III, LLC.
4.10#	Warrant issued by the Registrant on September 24, 2007 to Horizon Technology Funding Company V, LLC.
4.11#	Warrant issued by the Registrant on March 31, 2010 to Compass Horizon Funding Company LLC.

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4.12#	Warrant issued by the Registrant on March 31, 2010 to Oxford Finance Corporation.
4.13#	Form of Warrant to Purchase Series D-2 Preferred Stock issued by the Registrant to May 2011 Series D-2 preferred stock financing investors.
4.14#	Termination and Warrant Amendment Agreement dated May 18, 2012 among the Registrant and certain holders of Series D-2 preferred stock warrants.
4.15#	Second Warrant Amendment Agreement dated October 25, 2012 among the Registrant and certain holders of Series D-2 preferred stock warrants.
4.16#	Form of Warrant to Purchase Common Stock issued by the Registrant to October 2012 Series E preferred stock financing investors.
5.1	Opinion of Cooley LLP.
10.1+#	Form of Indemnity Agreement.
10.2+#	2011 Amended and Restated Equity Incentive Plan and forms of Stock Option Agreement thereunder, as amended.
10.3+#	2013 Equity Incentive Plan and form of Stock Option Agreement thereunder.
10.4+#	2013 Employee Stock Purchase Plan.
10.5+#	Employment Agreement dated November 9, 2011 between the Registrant and Michael A. Martino.
10.6+#	Amendment to Employment Agreement, dated January 12, 2012 between the Registrant and Michael A. Martino.
10.7+#	Letter Agreement, dated November 9, 2011 between the Registrant and Michael A. Martino.
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10.11+#	Letter Agreement dated January 20, 2012 between the Registrant and Athena Countouriotis, M.D.
10.12+#	Letter Agreement dated January 18, 2012 between the Registrant and Saiid Zarrabian.
10.13+#	Restated Board Compensation Letter Agreement dated April 1, 2011 between the Registrant and Faheem Hasnain.
10.14+#	Amendment to Restated Board Compensation Letter Agreement, dated January 12, 2012 between the Registrant and Faheem Hasnain.
10.15+#	Separation Agreement dated October 11, 2011 between the Registrant and Alan J. Lewis, Ph.D.
10.16+#	Separation Agreement dated January 25, 2012 between the Registrant and Christopher J. Morl.
10.17+#	Non-employee Director Compensation Policy.
10.18#	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Registrant and certain of its stockholders, as amended.
10.19#	Security Agreement dated March 31, 2010 between the Registrant and Oxford Finance Corporation.
10.20#	Venture Loan and Security Agreement dated March 31, 2010 among the Registrant, Compass Horizon Funding Company LLC and Oxford Finance Corporation.
10.21#	Lease Agreement dated July 22, 2004 between the Registrant and BMR-SORRENTO VALLEY LLC, as amended.

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10.22#	Lease Agreement dated July 17, 2012 between the Registrant and BMR-SORRENTO WEST LP.
10.23*#	Collaboration Agreement dated November 3, 2006 between the Registrant and Cephalon, Inc.
10.24*#	Exclusive License and Collaborative Research, Co-Development and Commercialization Agreement dated December 18, 2009 by and among the Registrant and Astellas Pharma Inc. and Astellas US LLC.
10.25*#	Collaboration Agreement dated September 14, 2010 between the Registrant and Genoptix, Inc.
21.1#	Subsidiaries of the Registrant.
23.1	Consent of Ernst & Young LLP, independent registered public accounting firm.
23.2	Consent of Cooley LLP. Reference is made to Exhibit 5.1.
24.1#	Power of Attorney. Reference is made to the signature page hereto.

\+ Indicates management contract or compensatory plan.
* Confidential treatment has been requested with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Securities and Exchange Commission.
\# Previously filed.

(b) Financial statement schedule.

II Valuation and qualifying accounts

No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or notes.

Item 17. ***Undertakings.***

The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, as amended, or the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

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SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of San Diego, State of California, on this 2nd day of May, 2013.

AMBIT BIOSCIENCES CORPORATION

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

President and Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Michael A. Martino, our President and Chief Executive Officer and Alan Fuhrman, our Chief Financial Officer, and each of them, as his true and lawful attorneys-in-fact and agents, each with the full power of substitution, for him and in his name, place or stead, in any and all capacities, to sign any and all amendments to this registration statement (including post-effective amendments), and to sign any registration statement for the same offering covered by this registration statement that is to be effective upon filing pursuant to Rule 462(b) promulgated under the Securities Act, and all post-effective amendments thereto, and to file the same, with 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 and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their, his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, as amended, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Michael A. Martino	President, Chief Executive Officer and Director	May 2, 2013
Michael A. Martino	(Principal Executive Officer)	
/s/ Alan Fuhrman	Chief Financial Officer (Principal Financial and Accounting Officer)	May 2, 2013
Alan Fuhrman		
/s/ Faheem Hasnain*		
Faheem Hasnain	Chairman of the Board, Director	May 2, 2013
/s/ David Bonita, M.D.*		
David Bonita, M.D.	Director	May 2, 2013
/s/ Steven A. Elms*		
Steven A. Elms	Director	May 2, 2013
/s/ Standish M. Fleming*		
Standish M. Fleming	Director	May 2, 2013
/s/ Allan P. Marchington, Ph.D.*	Director	May 2, 2013

Allan P. Marchington, Ph.D.

/s/ David R. Parkinson, M. D.*

David R. Parkinson, M. D.	Director	May 2, 2013

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Signature	Title	Date
/s/ Isai Peimer*		
Isai Peimer	Director	May 2, 2013
/s/ Joseph Regan*		
Joseph Regan	Director	May 2, 2013

* Pursuant to power of attorney

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

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* Confidential treatment has been requested with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Securities and Exchange Commission.
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