AMBIT BIOSCIENCES CORP
Form S-1/A
February 14, 2011
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As filed with the Securities and Exchange Commission on February 14, 2011

Registration No. 333-170413

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

AMENDMENT NO. 2

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	*(Primary Standard Industrial*	*(I.R.S. Employer*

incorporation or organization) *Classification Code Number)* *Identification Number)*

4215 Sorrento Valley Boulevard

San Diego, California 92121

(858) 334-2100

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

Alan J. Lewis, Ph.D.

President and Chief Executive Officer

Ambit Biosciences Corporation

4215 Sorrento Valley Blvd.

San Diego, California 92121

(858) 334-2100

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

Copies to:

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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 (2)**
Common Stock, $0.001 par value per share	$86,250,000	$6,150(3)

(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) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum offering price.

(3) 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 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 prospectus is not an offer to sell these securities and we are not soliciting offers to buy these securities in any state where the offer or sale is not permitted.

Subject to completion, dated February 14, 2011

Shares

COMMON STOCK

This is the initial public offering of common stock of Ambit Biosciences Corporation. We are selling shares of common stock. Prior to this offering, there has been no public market for our common stock. The initial public offering price of our common stock is expected to be between $ and $ per share.

We have applied for listing of our common stock on the Nasdaq Global Market under the symbol AMBT.

	Per Share	Total
Initial public offering price	**$**	**$**
Underwriting discounts and commissions	**$**	**$**
Proceeds to Ambit, before expenses	**$**	**$**

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

Investing in our common stock involves risks. See Risk Factors beginning on page 9.

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.

The underwriters expect to deliver the shares on or about , 2011.

J.P.Morgan **Credit Suisse**

Leerink Swann
Wedbush PacGrow Life Sciences

, 2011

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We have not authorized anyone to provide you with any information other than that contained in this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus may only be used where it is legal to offer and sell shares of our common stock. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock.

Until , 2011 (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.

For investors outside the United States: We have not and the underwriters have not done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the Risk Factors section beginning on page 9 and our consolidated financial statements and the related notes beginning on page F-1, before making an investment decision.

Overview

Ambit Biosciences Corporation is a biotechnology company engaged in discovering, developing and commercializing targeted small molecule therapeutics for the treatment of cancer. Our drug candidates are directed against an important family of enzymes called kinases, known to be involved in a range of human diseases. We are developing our lead drug candidate, quizartinib (formerly AC220), for the treatment of acute myeloid leukemia, or AML, under our global collaboration with Astellas Pharma Inc. and Astellas US LLC, collectively Astellas. Quizartinib is a once-daily, orally-administered, potent and selective kinase inhibitor currently in a pivotal Phase 2 clinical trial as monotherapy in relapsed/refractory AML. According to the American Cancer Society, approximately 13,000 adults were newly diagnosed with AML in 2009 in the United States with approximately 9,000 expected to die of the disease in that year. Quizartinib is being developed in concert with a companion diagnostic test to identify and treat the approximately one-third of AML patients with activating mutations in the FLT3 gene that drive a particularly aggressive and deadly form of this disease. We believe a targeted and personalized medicine approach to the treatment of AML has significant potential to improve patient outcomes and may transform what is an aggressive and deadly disease into a manageable condition. Novartis Gleevec (imatinib), a targeted kinase inhibitor, accomplished a similar transformation in the treatment of chronic myeloid leukemia. In addition to quizartinib, we have a pipeline of kinase inhibitors aimed at addressing significant unmet medical needs with potential advantages over existing therapeutics.

In November 2009, we initiated a single-arm, open-label pivotal Phase 2 clinical trial of quizartinib as monotherapy in relapsed/refractory AML patients. This trial is designed to evaluate the efficacy and safety of quizartinib in relapsed/refractory AML patients with internal tandem duplication, or ITD, mutations in the FLT3 gene, which we refer to as FLT3-ITD positive. ITD mutations account for the majority of activating mutations in FLT3. The study was amended on January 8, 2011 to also include a small cohort of AML patients without the FLT3-ITD mutation. We plan to enroll at least 300 patients worldwide and have enrolled 174 patients as of February 7, 2011. The trial is designed to measure the rate of complete response, or CR, complete response rate with incomplete platelet recovery, or CRp, complete response rate with incomplete neutrophil recovery, or CRi, and partial response, or PR. The co-primary endpoints of the trial are (1) composite complete response, or CR + CRp + CRi and (2) CR. Secondary endpoints include duration of remission, disease-free survival and overall survival.

Our pivotal Phase 2 clinical trial included an interim data analysis once the first 60 FLT3-ITD positive patients received at least one cycle of treatment, which occurred in September 2010. Clinical trial site-read data from this interim analysis of 53 evaluable patients has shown that 43.4% of these patients exhibited a composite complete response, consisting of 1.9% CRp and 41.5% CRi. An additional 28.3% exhibited a partial response. Median survival among these 53 evaluable patients was 24.4 weeks with 14 of these relapsed/refractory patients achieving responses that enabled a subsequent bone marrow transplant. We anticipate enrollment in the trial will be completed during the first half of 2011 and expect to report data within six months of completion of enrollment. If successful, this trial is expected to form the basis for a new drug application, or NDA, to be submitted to the U.S. Food and Drug Administration, or FDA, for the accelerated approval of quizartinib as monotherapy for relapsed/refractory AML patients.

In addition to our ongoing pivotal Phase 2 clinical trial of quizartinib in relapsed/refractory AML, we plan to initiate trials to evaluate the efficacy of quizartinib when combined with chemotherapy and subsequently as monotherapy maintenance in newly diagnosed AML patients. Since quizartinib is a potent inhibitor of a second receptor tyrosine kinase, KIT, we are also planning to explore the use of quizartinib as a treatment for certain solid tumors, including gastrointestinal stromal tumors, or GIST, and melanoma.

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Beyond quizartinib, we have a pipeline of kinase inhibitors in development for the treatment of various cancers. In October 2007, we licensed from Bristol-Myers Squibb Company, or BMS, exclusive worldwide rights to AC480, a once-daily, orally-administered, potent and selective inhibitor of the HER family of receptors. We are studying the oral formulation of AC480 in a Phase 1 clinical trial in patients with glioblastoma multiforme, or GBM. During the fourth quarter of 2010, we initiated a Phase 1 clinical trial for an intravenous, or IV, formulation of AC480 for the treatment of various solid tumors, including metastatic breast cancer and non-small cell lung cancer, or NSCLC. Also in the fourth quarter of 2010, we initiated a Phase 1 clinical trial for AC430 to determine the safety, tolerability and pharmacokinetics of AC430 in healthy volunteers. Our preclinical pipeline includes CEP-32496, a B-raf kinase inhibitor being developed by Cephalon.

Our integrated approach to drug discovery, combining our libraries of kinase-focused compounds and proprietary analytical tools with expertise in medicinal chemistry, molecular and cellular biology, pharmacology and pharmacokinetics, coupled with the panel of 442 kinase assays developed by us, accelerates our discovery and development of potent and selective kinase inhibitors. Since 2005, we have selected and advanced four kinase inhibitor drug candidates into preclinical and clinical development: quizartinib, AC480, AC430 and CEP-32496.

Our Pipeline of Targeted Therapies

The following table summarizes the status of our product pipeline:

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Our Strategy

Our objective is to develop and commercialize products that treat serious unmet medical needs in patients suffering from cancer. The key components of our business strategy are:

- Develop and commercialize our lead drug candidate, quizartinib, in AML and certain solid tumors in partnership with Astellas.
- Advance our pipeline of clinical and preclinical drug candidates.
- Establish strategic partnerships to accelerate development timelines and maximize the commercial potential of our drug candidates.
- Leverage our discovery capabilities and our understanding of the human kinome to be a leading company in the discovery and development of targeted kinase drugs.
- Build capabilities to allow us to effectively commercialize our drug candidates.

Our Collaboration with Astellas

In December 2009, we entered into a worldwide agreement with Astellas to jointly research, develop and commercialize FLT3 kinase inhibitors. As partial consideration for the exclusive license rights granted to Astellas, we received an upfront payment of $40.0 million. In addition, we may receive payments of up to $350.0 million upon the achievement of development and regulatory milestones. We are also entitled to receive tiered double-digit royalty payments on sales as well as annual sales-based milestones. The agreement provides that we and Astellas will conduct a joint five-year research program related to certain designated follow-on compounds to quizartinib. We share development costs in the United States and European Union and research costs on follow-on compounds equally with Astellas. Astellas is responsible for all other development costs and costs associated with commercialization of products covered by the agreement. We retain the right to co-promote and share profits with Astellas on both quizartinib and any follow-on drugs in the United States.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the Risk Factors section of this prospectus beginning on page 9. In particular:

- We are dependent on the success of our lead product candidate, quizartinib, which is still in clinical development, and we cannot give any assurance that it, or any other product candidates, will receive regulatory approval, which is necessary before they can be commercialized.
- We share oversight of the development of quizartinib globally with Astellas and therefore depend upon Astellas in our efforts to obtain regulatory approval and to commercialize quizartinib.
- 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.

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

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

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.

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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 product candidates, or continue our research and development programs.

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 4215 Sorrento Valley Blvd., 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.

Unless the context indicates otherwise, as used in this prospectus, the terms Ambit, Ambit Biosciences we, us and our refer to A Biosciences Corporation, a Delaware corporation, and its subsidiaries taken as a whole, unless otherwise noted.

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.

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THE OFFERING

Common stock offered	shares
Common stock to be outstanding after this offering	shares
Over-allotment option	shares
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $ million, or approximately $ million if the underwriters exercise their over-allotment option in full, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We expect to use the net proceeds from this offering to fund development and commercialization of quizartinib, our lead product candidate, to fund the development of our other product candidates and for working capital and other general corporate purposes. See Use of Proceeds on page 37 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 9 for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Proposed Nasdaq Global Market symbol	AMBT

The number of shares of our common stock to be outstanding after this offering is based on shares of common stock outstanding as of October 31, 2010 after giving effect to the conversion of all of our convertible preferred stock into common stock upon the closing of this offering and excludes:

- 5,839,779 shares of common stock issuable upon exercise of stock options outstanding as of October 31, 2010 at a weighted-average exercise price of $1.14 per share;
- an aggregate of 452,470 shares of common stock reserved for future issuance under the predecessor plan to our 2011 amended and restated equity incentive plan (referred to herein as the 2011 pre-IPO plan) as of October 31, 2010 and an aggregate of additional shares of common stock that will be available under our new 2011 equity incentive plan (referred to herein as our 2011 post-IPO plan) and our 2011 employee stock purchase plan, each of which will be adopted upon the closing of this offering; and
- 3,189,163 shares of common stock issuable upon the exercise of warrants outstanding as of October 31, 2010 at a weighted-average exercise price of $1.79 per share.

Unless otherwise noted, the information in this prospectus assumes:

a - for - reverse stock split of our common stock to be effected prior to the closing of this offering;

the filing of our restated certificate of incorporation and the adoption of our restated bylaws as of the closing date of this offering;

no exercise by the underwriters of their option to purchase shares of common stock to cover over-allotments;

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the issuance by us of 1,538,461 shares of our Series C-2 redeemable convertible preferred stock and 612,649 shares of Series D redeemable convertible preferred stock prior to the closing of this offering upon exercise of a put right held by GrowthWorks Canadian Fund Ltd., or the GrowthWorks put right;

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 24,608,183 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 649,573 shares of common stock upon the closing of this offering;

the issuance of shares of our common stock upon the closing of this offering as a result of the automatic conversion and/or cancellation of $15.0 million of secured notes (and accrued interest thereon) that we and Ambit Canada issued in September 2010 (including the shares issuable upon the automatic exercise of a warrant that we issued in September 2010 in connection with the issuance by Ambit Canada of a portion of the secured notes), assuming an initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) and assuming the conversion and/or cancellation occurs on , 2011 (for purposes of calculating the accrued interest on the notes).

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SUMMARY CONSOLIDATED FINANCIAL INFORMATION

The following summary consolidated financial information should be read together with our financial statements and accompanying notes and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. The summary financial data in this section are not intended to replace our financial statements and the related notes. Our historical results are not necessarily indicative of our future results.

The summary statement of operations data for the years ended December 31, 2007, 2008 and 2009 are derived from our audited financial statements appearing elsewhere in this prospectus. The summary statement of operations data for the nine months ended September 30, 2009 and 2010 are derived from our unaudited financial statements appearing elsewhere in this prospectus. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements included in this prospectus and include, in our opinion, all adjustments, consisting of normal recurring adjustments necessary for the fair presentation of the financial information in those statements.

	Years Ended December 31,			Nine Months Ended September 30,	
	2007	2008	2009	2009	2010
				(unaudited)	
	(in thousands except share and per share data)				
Statement of Operations Data:					
Revenues:					
Collaboration arrangements	$ 3,621	$ 3,621	$ 3,466	$ 2,716	$ 14,782
Kinase profiling services (held-for-sale)	10,692	24,480	14,647	10,677	5,229
Total revenues	14,313	28,101	18,113	13,393	20,011
Operating expenses:					
Research and development	19,386	26,884	29,280	20,371	29,155
General and administrative	6,466	6,581	5,788	4,134	6,294
In-process research and development	25,000				
Cost of kinase profiling services revenue (held-for-sale)	2,993	4,194	3,777	2,888	1,298
Total operating expenses	53,845	37,659	38,845	27,393	36,747
Loss from operations	(39,532)	(9,558)	(20,732)	(14,000)	(16,736)
Other income (expense):					
Interest expense	(1,874)	(1,736)	(4,899)	(2,319)	(9,676)
Other income (expense)	946	1,202	(364)	(278)	(7)
Change in fair value of redeemable convertible preferred stock warrant liabilities	278	258	(658)	(243)	337
Total other income (expense)	(650)	(276)	(5,921)	(2,840)	(9,346)
Loss before income taxes	(40,182)	(9,834)	(26,653)	(16,840)	(26,082)
Provision for (benefit from) income taxes	196		(191)		1,900
Consolidated net loss	(40,378)	(9,834)	(26,462)	(16,840)	(27,982)
Net loss attributable to redeemable non-controlling interest	411	86	2,177	1,245	1,446
Net loss attributable to Ambit Biosciences Corporation	(39,967)	(9,748)	(24,285)	(15,595)	(26,536)
Accretion to redemption value of redeemable convertible preferred stock	(3,867)	(61)	(61)	(46)	(626)
Change in fair value of redeemable non-controlling interest	(180)	1,737	(7,567)	(3,384)	702
Net loss attributable to common stockholders	$ (44,014)	$ (8,072)	$ (31,913)	$ (19,025)	$ (26,460)

Net loss per share attributable to common stockholders, basic and diluted[1]	$ (47.30)	$ (8.38)	$ (15.47)	$ (11.39)	$ (8.15)
Weighted-average shares outstanding, basic and diluted[1]	930,465	963,390	2,063,489	1,671,012	3,247,170
Pro forma net loss per share, basic and diluted (unaudited)[1]			$ (1.37)		$
Weighted-average pro forma shares outstanding, basic and diluted (unaudited)[1]			18,828,136		

(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 and the number of shares used in computation of the per share amounts.

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The following table sets forth our summary balance sheet data as of September 30, 2010 (unaudited):

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 and 612,649 shares of Series D redeemable convertible preferred stock prior to the closing of this offering upon 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 issued upon the exercise of the GrowthWorks put right, into an aggregate of 24,608,183 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 649,573 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;

(4) the issuance of shares of our common stock upon the closing of this offering as a result of the automatic conversion and/or cancellation of $15.0 million of secured notes (and accrued interest thereon) that we and Ambit Canada issued in September 2010 (including the shares issuable upon the automatic exercise of a warrant that we issued in September 2010 in connection with the issuance by Ambit Canada of a portion of the secured notes), assuming an initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) and assuming the conversion and/or cancellation occurs on , 2011 (for purposes of calculating the accrued interest on the notes).

on a pro forma as adjusted basis to additionally give effect to the sale of shares of common stock in this offering, assuming an initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus), after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

	As of September 30, 2010		
	Actual	Pro Forma	Pro Forma as Adjusted
	(unaudited, in thousands)		
Balance Sheet Data:			
Cash and cash equivalents	$ 37,318	$	$
Working capital	26,276		
Total assets	48,086		
Redeemable convertible preferred stock warrant liabilities	1,513		
Redeemable non-controlling interest	9,041		
Derivative liability- conversion feature	885		
Notes payable	24,144		
Redeemable convertible preferred stock	96,488		
Convertible preferred stock	13,752		

Accumulated deficit	(167,099)
Total stockholders deficit	(143,307)

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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 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, operating results and financial condition 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 dependent on the success of our lead product candidate, quizartinib, which is still in clinical development, and we cannot give any assurance that it, or any other product 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 product candidate, which is currently in a pivotal Phase 2 clinical trial. Our other drug candidates are in earlier stages of development. Our business depends entirely on the successful development and commercialization of our product 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 and significant marketing efforts before we generate any revenues from product sales. The United States Food and Drug Administration, or FDA, has also informed us that an approved companion diagnostic is required in order to support the approval of quizartinib. We are not permitted to market or promote quizartinib, or any other product candidates, before we receive regulatory approval from the FDA, or comparable foreign regulatory authorities.

We expect to file for initial regulatory approval of quizartinib for the treatment of certain patients with acute myeloid leukemia, or AML, based on our current pivotal Phase 2 clinical trial and our planned initiation of a Phase 3 clinical trial in the second half of 2011. 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 foreign authorities, for quizartinib or received marketing approval for quizartinib, and we cannot be certain that this product candidate will be successful in clinical trials or receive regulatory approval. If we do not receive regulatory approvals and successfully commercialize quizartinib, 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, upon 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, with our partners Astellas Pharma Inc. and Astellas US LLC, or collectively Astellas, plan to seek regulatory approval to commercialize quizartinib both in the United States and in some 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.

We share oversight of the development of quizartinib globally with Astellas and therefore depend upon Astellas in our efforts to obtain regulatory approval and to commercialize quizartinib.

We jointly research and develop FLT3 kinase inhibitors, including quizartinib, in oncology and non-oncology indications with Astellas. Astellas plays a significant role in the conduct of the clinical trials and the subsequent collection and analysis of data. Employees of Astellas are not our employees, and we have limited ability to control the amount of time or resources they devote to quizartinib or other compounds covered by our collaboration. If Astellas is unable to perform in a manner consistent with the standard contemplated by our agreement, it may delay the potential approval of our regulatory applications as well as the potential commercialization and manufacturing of quizartinib. A material breach by Astellas of our collaboration

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agreement may also delay potential regulatory approval and commercialization of quizartinib. Moreover, although we have non-compete restrictions in place with Astellas, Astellas may have relationships with other commercial entities, some of which may compete with us. Astellas may also elect to focus its resources and priorities on other programs that it is pursuing rather than on quizartinib. If Astellas assists our competitors or fails to adequately support the quizartinib program, it could harm our competitive position.

We will rely on Genoptix, Inc. to obtain marketing clearance or approval of the companion diagnostic test for quizartinib. There is no guarantee that the FDA will grant timely clearance or approval of this test, if at all, and failure to obtain such timely clearance or approval would adversely affect our ability to obtain approval for quizartinib.

We are initially seeking approval of quizartinib in relapsed/refractory AML patients with internal tandem duplication, or ITD, mutations in the 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, the pivotal trial designed to obtain marketing approval for quizartinib uses a diagnostic test to select patients that are FLT3-ITD positive. In the United States, the FDA requires that the diagnostic test used to select patients in a pivotal trial be approved in parallel with the drug candidate as a companion diagnostic. As a result, we believe it will be critical to the approval of quizartinib to develop a companion genetic test to test for the FLT3-ITD positive genotype. Companion diagnostic tests are subject to regulation by the FDA and may, in the future, become subject to regulation by comparable agencies in various foreign countries. The process of complying with the requirements of the FDA and possibly of comparable agencies is costly, time consuming and burdensome.

We have entered into an agreement with Genoptix, Inc., or Genoptix, pursuant to which Genoptix will be responsible for determining the appropriate regulatory pathway for the companion diagnostic and obtaining market clearance or approval from the FDA. Based on FDA guidance, Genoptix will need to submit a Pre Market Approval application, or PMA, for such test, which we anticipate will happen in parallel with our submission of an NDA for quizartinib. We do not believe that any clinical trials other than the quizartinib pivotal trial will be required for the companion diagnostic test PMA. 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 clinical trial sites allow us to permit a third-party to test these samples, as required.

Despite the time and expense expended, regulatory clearance or approval is never guaranteed. If regulatory clearance or approval is delayed, or if Genoptix is unable to obtain FDA approval of the companion diagnostic test at all or in parallel with the approval of quizartinib, or is unable to commercialize the test successfully and in a manner that effectively supports our commercial efforts, or if the information concerning the differential response to quizartinib resulting from certain genetic variation is not included in the approved label for quizartinib, the commercial launch of quizartinib may be significantly and adversely affected.

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 uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product 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.

We may experience delays in clinical trials of our product candidates. Quizartinib has completed a Phase 1/2 clinical trial for the treatment of AML. We initiated a pivotal Phase 2 clinical trial of quizartinib in patients with relapsed/refractory AML in the fourth quarter of 2009 and anticipate completing enrollment in the first half of

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2011. We anticipate that enrollment in this trial will be completed during the first half of 2011 and expect to report data within six months of completion of enrollment. We plan to initiate additional clinical trials in AML and other indications. We do not know 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 site;
- recruiting suitable patients to participate in a trial;
- having patients complete a trial or return for post-treatment follow-up;
- clinical sites deviating from trial protocol or dropping out of a trial;
- adding new sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

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

We could encounter delays if prescribing physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product 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 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, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product 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, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product 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, 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. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product s clinical development. We have not obtained regulatory approval for any product candidate.

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Our product 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 product candidate is safe and effective for any 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 product 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 product candidates may not be sufficient to support the submission of an NDA or other submission or to obtain regulatory approval in the United States or elsewhere;
- 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 may fail to approve the PMA for the companion diagnostic, and this may apply in other jurisdictions, if applicable; 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.

Based on our consultation with the FDA and in light of the serious unmet need for the treatment of AML, we designed our current quizartinib pivotal Phase 2 clinical trial for AML patients with a FLT3 mutation as an open-label trial to be used as a registration trial for NDA approval. An open-label trial allows for rapid patient enrollment and therefore a potentially faster regulatory approval process. However, open-label studies carry with them certain regulatory risks. In particular, results are determined based on the qualitative judgment of the FDA rather than pure statistics and the FDA s acceptance of the trial results to support NDA approval is uncertain.

We may be required to suspend or discontinue clinical trials due to adverse side effects or other safety risks that could preclude approval of quizartinib or any of our future product candidates.

Our clinical trials may be suspended or terminated at any time by us, our collaborators, institutional review boards, the FDA or other regulatory authorities for a number of reasons, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, unforeseen safety issues or adverse side effects for participants, failure to demonstrate a benefit from using the investigational drug, or negative or equivocal findings of the Data Safety Monitoring Board, or DSMB, or the institutional review board for a clinical trial. If we elect or are forced to suspend or terminate any clinical trial of any product candidates that we develop, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product candidates will be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

To date, patients treated with quizartinib have experienced drug-related side effects including nausea, diarrhea, dysgeusia (the distortion of the sense of taste), peripheral edema (swelling of the legs), fever, vomiting, anemia, fatigue, headache, and abdominal pain. In addition, changes in ECG pattern called QTc prolongation have been observed. Such QTc 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. Results of our trials could reveal a high and unacceptable severity and

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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 product candidates for any or all targeted indications. 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, financial condition and prospects significantly.

Additionally if quizartinib receives marketing approval, and 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 quizartinib;
- 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 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 and could significantly harm our business, results of operations and prospects.

If we, along with our partner, Astellas, fail to gain and maintain approval from regulatory authorities in international markets for quizartinib and any future product candidates for which we have rights in international markets, our market opportunities will be limited and our business will be adversely impacted.

Sales of our product candidates outside of the United States 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 our 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 product is also subject to approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and our partner, Astellas, and could delay 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. In addition, the failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in others. None of our product candidates is approved for sale in any international market. If Astellas fails to comply with regulatory requirements in our international markets or to obtain and maintain required approvals, our target market will be reduced and our ability to generate revenues will be diminished, which would significantly harm our business, results of operations and prospects.

If we are unable to obtain FDA approval of our product 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 product candidates in the United States. If we fail to obtain FDA approval to market our product candidates, we will be unable to sell our product 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 product 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 product candidate is both safe and

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effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the approval requirements typically takes several years and the time needed to satisfy them may vary substantially, based on the type, complexity and novelty of the pharmaceutical product. We cannot predict if or when we might receive regulatory approvals for any of our product 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 product 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 for approval or even to make our applications approvable. 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 and prospects.

Even if we do receive regulatory approval to market a product 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 product candidates or any product 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 product candidates, generating revenues and achieving and sustaining profitability.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing FDA obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product 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 product 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 product candidate. In addition, if the FDA approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion 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 and good clinical practices 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;

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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 product 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.

We and our collaborators rely on third parties to conduct our preclinical and 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 product candidates and our business could be substantially harmed.

We have agreements with third-party CROs to provide monitors for and to manage data for our ongoing preclinical and clinical programs. We rely heavily on these parties for execution of our preclinical and clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol. We and our CROs are required to comply with current good clinical practices, which are regulations and guidelines enforced by the FDA for all of our products in clinical development. The FDA enforces these good clinical practices regulations through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fails to comply with applicable good clinical practices regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our clinical trials comply with good clinical practices regulations. In addition, our clinical trials must be conducted with product produced under good manufacturing practices regulations, and will require a large number of test subjects. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

Our CROs have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us if it can be reasonably demonstrated that the safety of the subjects participating in our clinical trials warrants such termination, if we make a general assignment for the benefit of our creditors or if we are liquidated.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. 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, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding additional 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, financial condition and prospects.

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We will rely on Genoptix to develop the quizartinib companion diagnostic test and the product sales and profitability of quizartinib will suffer if Genoptix fails to do so.

We have contracted with Genoptix to develop a companion diagnostic test for quizartinib. If Genoptix or its third-party suppliers were to cease or interrupt production of or otherwise fail to perform the companion diagnostic test, or the materials required to perform it, in a timely manner or at all, we could be unable to obtain a replacement laboratory for an indeterminate period of time. This could adversely affect our ability to satisfy demand for quizartinib, which could cause product sales and profitability of quizartinib to suffer and could have an adverse effect on our business, financial condition and prospects.

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 product candidate, and our commercialization of any of our product candidates, including quizartinib, could be stopped, delayed or made less profitable if those third parties fail to obtain approval of the FDA, fail to provide us with sufficient quantities of drug product or fail to do so at acceptable quality levels or prices.

The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be completed after we submit our NDA 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 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 and/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 does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates.

We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product 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 product candidate to complete the clinical trial, any significant delay in the supply of a product 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 product candidates. If our manufacturers or we are unable to purchase these raw materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product 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 product candidates.

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

We have not entered into long-term agreements with our current contract manufacturers or with any alternate fill/finish suppliers, and though 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.

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We believe we 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 product candidate quizartinib does not guarantee faster approval.

We received Fast Track designation for our product candidate quizartinib for the treatment of AML. Fast track designation is a process designed to facilitate the development and expedite the review of new drugs to treat serious or life-threatening conditions and that have the potential to address an unmet medical need. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. For a Fast Track product, the FDA may consider for review on a rolling basis sections of the NDA before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA. 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 drug products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenues.

We currently do not have a sales 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 our own sales force to promote quizartinib in the United States, together with Astellas. However, the establishment and development of our own 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 collaborators and co-promoters. 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 product candidates.

In addition to being reliant on Astellas to co-commercialize quizartinib in the United States, if appropriate regulatory approvals are obtained, we will be reliant on Astellas for commercializing quizartinib in international markets. In the event Astellas fails to adequately commercialize quizartinib because it fails to gain regulatory approvals, lacks adequate financial or other resources or decides to focus on other initiatives, our ability to successfully commercialize quizartinib would be limited, which would adversely affect our business, financial condition, results of operations and prospects.

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

A key element of our strategy is to commercialize a portfolio of other product candidates in addition to quizartinib. As a significant part of our growth strategy, we intend to develop and commercialize additional

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products and product candidates through our research program using our scientific expertise and experience in kinase drug discovery. The success of this strategy depends upon our ability to identify, select and acquire pharmaceutical product candidates and products that fit into our development plans on terms that are acceptable to us.

Any product candidate we identify will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, extensive clinical trials and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product 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 disease targets and product candidates require substantial technical, financial and human resources whether or not we ultimately identify any candidates. If we are unable to develop suitable potential product candidates through internal research programs or by obtaining rights to novel therapeutics from third parties, our business and prospects will suffer.

We cannot be certain that our product 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 product candidates other than quizartinib. In addition, we have limited clinical data with respect to any of these other potential product candidates. Even if we are successful in completing 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 product candidates to follow quizartinib, and our business prospects would be harmed significantly.

Our commercial success depends upon attaining significant market acceptance of our product 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 product 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 product candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety as demonstrated in clinical trials;
- the clinical indications for which the drug 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 product candidates over alternative treatments;
- the safety of product candidates seen in a broader patient group, including its use outside the approved indications;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third parties and government authorities;

relative convenience and ease of administration;

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the prevalence and severity of adverse side effects; and

the effectiveness of our sales and marketing efforts.

If our approved drugs fail to achieve market acceptance, we will not be able to generate significant revenues.

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. 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 commercially available kinase inhibitors such as Bayer and Onyx s Nexavar (sorafenib) and Pfizer s Sutent (sunitinib), two multi-kinase inhibitors that inhibit the FLT3 kinase 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 PKC-412.

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

discover and develop medicines 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 of our competitors products could limit the demand, and the price we are able to charge, for quizartinib. 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, financial condition and prospects.

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

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

We and our partner, Astellas, intend to seek approval to market our future products in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions, we will be subject to rules and regulations in those jurisdictions relating to our product. In some foreign countries, particularly 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 product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for any of our product 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 supporting scientific, clinical and cost-effectiveness data for the use of our products to the payor. 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 Heathcare 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.

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 products 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;

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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. 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 any future product candidates and programs because our research and development pipeline may be insufficient, our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort and/or third parties may not view our product 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 product candidates could also delay the development and commercialization of our product 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, Dr. Alan J. Lewis, our Executive Vice President of Research and Development, Dr. Wendell Wierenga, our Chief Operating Officer, Christopher J. Morl and our Chief Medical Officer and Senior Vice President, Clinical Development, Dr. Robert Corringham. In order to induce valuable employees to remain at Ambit, we have provided incentive stock options that vest over time. The value to employees of stock options that vest over time is 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. As a result, currently there is a shortage of experienced scientists, which is likely to continue. Competition for skilled personnel in our market is very intense and competition for experienced scientists may limit our ability to hire and retain highly qualified personnel on acceptable terms.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with all of our employees, these employment arrangements 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, financial condition and prospects. 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 also may provide more diverse opportunities and better chances for career advancement. Some

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of these characteristics may be more appealing to high quality candidates than what we have to offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize drug candidates will be limited.

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

As of December 31, 2010, we employed 75 employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial and other resources. Future growth would impose significant added responsibilities on members of management, including:

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

Our management may also have to divert a disproportionate amount of its attention away from day-to-day activities and devote a substantial amount of time to managing these growth activities. Our future financial performance and our ability to commercialize quizartinib and other product candidates will depend, in part, on our ability to effectively manage any future growth.

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. Our corporate headquarters is located in California and other critical business operations and some of our suppliers are 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 product candidates internationally could materially adversely affect our business.

If approved for commercialization, we expect quizartinib to be marketed worldwide. 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 and 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;

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- 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;
- workforce uncertainty in countries where labor unrest is more common than in 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, provide accurate information to the FDA, comply with manufacturing standards we have established, comply with federal and state health-care fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, 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.

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

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

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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 revenues; and

the inability to commercialize our product candidates.

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. 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, biological and radioactive materials. In addition, our operations produce medical, radioactive and hazardous waste products. Federal, state and local laws and regulations in the United States govern the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our procedures for use, 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, financial condition and prospects.

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. 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. 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 have incurred significant operating losses since our inception, including consolidated net losses of $40.4 million, $9.8 million and $26.5 million for the years ended December 31, 2007, 2008 and 2009, respectively and $16.8 million and $28.0 million for the nine months ended September 30, 2009 and 2010, respectively. As of September 30, 2010, we had an accumulated deficit of $167.1 million. We do not know whether or when we will become profitable. 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

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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 product candidate, quizartinib.

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 would 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 upon our ability to generate revenues from drug sales. To date, we have no products approved for commercial sale and have not generated any revenues from drug sales and we may never be able to develop marketable drugs. Thus far, substantially all of our revenues have been generated from fees for research services, from license or collaboration agreements and from our screening business which we sold in October 2010. We do not anticipate generating revenues, if any, from sales of quizartinib until 2012 at the earliest and we will never generate revenues from quizartinib if we do not obtain regulatory approval. Our ability to generate future revenues depends heavily on our success in:

- developing and securing United States and/or foreign regulatory approvals for quizartinib;
- commercializing quizartinib and any other product candidates for which we receive approval; and
- generating a pipeline of innovative product candidates utilizing 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.

If we fail to obtain additional financing, we may be unable to complete the development and commercialization of quizartinib or other product 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 and other product candidates;
- launch and commercialize any product candidates for which we receive regulatory approval, including building our own sales force to address certain markets; and
- continue our research and development programs to advance our internal product pipeline.

We estimate that our net proceeds from this offering will be approximately $ million, based upon an assumed initial public offering price of $ per share (the midpoint of the price range set forth on the cover of this prospectus) after deducting underwriting discounts and commissions and offering expenses payable by us. We expect that the net proceeds from this offering, together with our existing cash and cash equivalents will be sufficient to fund our capital requirements for at least the next 12 months including estimated expenditures of approximately $45.2 million on the development of quizartinib through the submission of an NDA and the initiation of the confirmatory Phase 3 clinical trial required by the FDA. We will require additional capital for the further development and commercialization of our lead product candidate quizartinib and may also 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. 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 product candidates or one or more of our other research and development initiatives. We also could be required to:

significantly delay, scale back or discontinue the development or commercialization of quizartinib or our other clinical and preclinical programs;

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seek collaborators for one or more of our current or future product 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 product candidates that we otherwise would seek to develop or commercialize ourselves; or

license or acquire additional product candidates.

Any of the above events could significantly harm our business, financial condition and prospects.

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

We may seek additional capital through a combination of private and public 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 also result in certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us.

The timing of the milestone and royalty payments we are required to make to BMS are uncertain and could adversely affect our business, financial condition and prospects.

We are party to license agreements with Bristol-Myers Squibb Company, or BMS, pursuant to which we acquired an exclusive license to certain intellectual property related to our AC480 product candidate. We are obligated to make certain cash milestone payments to BMS upon completion of certain development milestones and the receipt of certain regulatory approvals of such product candidate. In addition, we are required to make certain cash royalty payments upon our achievement of target levels of commercial sales of such product candidate. The timing of our achievement of the events that trigger milestone payments to BMS are subject to factors relating to the preclinical, clinical and regulatory development and commercialization of the programs, many of which are beyond our control. Though we believe that these royalty rates and milestone payments are reasonable in light of our business plan, we will require large amounts of capital to satisfy these obligations. We may become obligated to make a milestone payment when we do not have the cash on hand to make such payment, which could require us to delay our clinical trials, curtail our operations, scale back our commercialization and marketing efforts or seek funds to meet these obligations on terms unfavorable to us. In addition, if we are unable to make any payment when due or if we fail to use commercially reasonable efforts to achieve certain development and commercialization milestones within the timeframes required by our license agreements, the other parties may have the right to terminate the agreement and all of our rights to develop and commercialize the associated product candidate.

We are substantially dependent on milestone and other payments due to us under our collaboration agreements with our partners.

Pursuant to the collaboration agreement we entered into with Astellas in December 2009, we and Astellas share oversight of the research and development of quizartinib and other FLT3 kinase inhibitor candidates and are each obligated to use commercially reasonable efforts to perform the tasks and activities assigned to us under each research and development plan. Astellas paid us an upfront, non-refundable fee of $40.0 million, and upon the successful achievement of clinical development and regulatory milestones, we are eligible to receive from Astellas up to an additional $350.0 million. Further, we are entitled to receive from Astellas tiered, double-digit

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royalty payments calculated as a percentage of aggregate net sales and additional annual sales milestone payments. If quizartinib or the other collaboration compounds fail to meet clinical development or regulatory milestones or if we or Astellas fail to meet our respective obligations under the agreement our ability to achieve the milestones may be impacted and potential milestone payments due to us may be delayed or forfeited which would materially adversely affect our business, operating results and financial condition.

Pursuant to the collaboration agreement we entered into with Cephalon in November 2006 we licensed collaboration compounds including CEP-32496 to Cephalon. We have received a $1.0 million milestone payment under the agreement to date and we may be entitled to receive up to $46.5 million in additional payments upon the achievement of certain development, regulatory and sales milestones along with tiered royalty payments calculated as a percentage of net sales of the collaboration compounds. If the collaboration compounds fail to meet development, regulatory or sales milestones or if we or Cephalon fail to meet our respective obligations under the agreement, we may not receive the milestone payments which would adversely impact our business, operating results and financial condition.

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, 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 initial public offering, 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, 2009, we had federal and state net operating loss carryforwards of approximately $91.9 million and $63.0 million, respectively, that 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 been experiencing 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 the recent economic downturn and volatile business environment and continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate further, 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 September 30, 2010, we had $37.3 million of cash and cash equivalents. While as of the date of this prospectus, we are not aware of any downgrades, material losses, or other significant deterioration in the fair value of our cash equivalents or marketable securities since September 30, 2010, no assurance can be given that further deterioration in conditions 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. Further dislocations in the credit market may adversely impact the value and/or liquidity of marketable securities owned by us.

There is a possibility that our stock price may decline, due in part to the volatility of the stock market and the general economic downturn.

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Risks Related to Our Intellectual Property

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

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

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 Cephalon and Astellas, 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 product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product 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, an interference proceeding can be provoked by a third-party or instituted by the United States Patent and Trademark Office, or 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.

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 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. Further, 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 material disclosure of the intellectual property related to our technologies to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

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

Our commercial success depends in part on our and our collaborators avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including interference and reexamination proceedings before the U.S. PTO or oppositions and other comparable proceedings in foreign jurisdictions. Numerous United States 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.

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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 product 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 product 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 use in quizartinib, as well as a third party patent related to diagnostic testing for certain FLT3 mutations. 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 obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product 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 product 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 product candidates, which could harm our business significantly.

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

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing.

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

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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.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of entities that disclose such information to us as part of our past providing screening services or of other third parties.

Prior to our sale of our profiling services screening business in October 2010, customers for our screening services provided confidential and proprietary information to us for screening. 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 our customers or our employees former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial cost and be a distraction to our management and other 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 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. The initial public offering price for our common stock was 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. This initial public offering price may vary from the market price of our 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. In addition to the factors discussed in this Risk Factors section and elsewhere in this prospectus, these factors include:

- 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;
- failure to meet or exceed revenues and financial projections we provide to the public;
- actual or anticipated variations in quarterly operating results;
- failure to meet or exceed the estimates and projections of the investment community;

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overall performance of the equity markets;

adverse regulatory decisions;

adverse results or delays in clinical trials;

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

our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;

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

developments concerning our collaborations, including but not limited to those with our sources of manufacturing supply;

the termination of a collaboration or the inability to establish additional collaborations;

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

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 maintain an adequate rate of growth;

our ability to effectively manage our growth;

our ability to successfully enter new markets;

disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

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

termination of our collaboration agreement by Astellas;

additions or departures of key scientific or management personnel;

issuances of debt or equity securities;

significant lawsuits, including patent or stockholder litigation;

changes in the market valuations of similar companies;

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

trading volume of our common stock;

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

ineffectiveness of our internal controls;

general political and economic conditions;

effects of natural or man-made catastrophic events; and

other events or factors, many of which are beyond our control.

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In addition, the stock market in general, and The Nasdaq Global Market and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market price of a company s securities, securities class action litigation has often been instituted against companies. 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, our ability to pay cash dividends is currently prohibited by the terms of one of our debt financing arrangements, 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.

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 % of our voting stock and, upon the closing of this offering, that same group will hold approximately % of our outstanding voting stock (assuming no exercise of the underwriters over-allotment option) in each case assuming an initial public offering price of $ per share (the midpoint of the range set forth on the cover page of this prospectus) and a conversion date of 2011 (for purposes of calculating the accrued interest on the notes to be converted into shares of common stock and the number of shares to be issued upon the automatic exercise of a warrant to purchase common stock). 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 $ per share, based on an initial public offering price of $ per share. Further, investors purchasing common stock in this offering will contribute approximately % of the total amount invested by stockholders since our inception, but will own only approximately % of the shares of common stock outstanding after giving effect to this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less than the price offered to the public in this offering when they purchased their shares and the exercise of stock options granted to our employees. In addition, as of October 31, 2010, options to purchase 5,839,779 shares of our common stock at a weighted-average exercise price of $1.14 per share and warrants exercisable for up to 3,189,163 shares of our common stock at a weighted-average price of $1.79 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

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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 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. In addition, the Sarbanes-Oxley Act of 2002, 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. We expect these rules and regulations 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.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we will be required to perform system and process evaluation and testing of our internal controls over financial reporting to allow management and our independent registered public accounting firm to report, commencing in our annual report on Form 10-K for the year ending December 31, 2012, on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our testing, or the subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses. Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge. Moreover, if we are not able to comply with the requirements of Section 404 in a timely manner or if we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities, which would require additional financial and management resources.

New laws and regulations as well as changes to existing laws and regulations affecting public companies, including the provisions of the Sarbanes-Oxley Act of 2002 and rules adopted by the SEC and by Nasdaq, would likely result in increased costs to us as we respond to their requirements.

Sales of a substantial number of shares of our common stock 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 September 30, 2010, upon the closing of this offering, we will have outstanding a total of 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 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

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restriction, in the public market immediately following this offering. J.P. Morgan Securities LLC and Credit Suisse Securities (USA) 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 (subject to extension upon the occurrence of specified events). After the lock-up agreements expire, up to an additional shares of common stock will be eligible for sale in the public market, 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 $ per share (the midpoint of the range set forth on the cover page of this prospectus) and a conversion date of , 2011 (for purposes of calculating the accrued interest on the notes to be converted into shares of common stock and the number of shares to be issued upon the automatic exercise of a warrant to purchase common stock). In addition, 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 shares of our common stock, or % of our total outstanding common stock as of September 30, 2010, will be entitled to rights with respect to the registration of their shares under the Securities Act of 1933, as amended (or the Securities Act), subject to the 180-day lock-up agreements described above and assuming an initial public offering price of $ per share (the midpoint of the range set forth on the cover page of this prospectus) and a conversion date of , 2011 (for purposes of calculating the accrued interest on the notes to be converted to shares of common stock and the number of shares to be issued upon the automatic exercise of a warrant to purchase common stock). 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. 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 will be needed in the future to continue our planned operations. 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 those of holders of our common stock, including shares of common stock sold in this offering.

Pursuant to our 2011 equity incentive plan, or 2011 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 2011 post-IPO plan will automatically increase each year by an amount equal to % 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.

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

Our management will have broad discretion in the application of the net proceeds from this offering, and you will be relying on the judgment of our management regarding the application of these proceeds. 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 to fund clinical trials and other research and development activities for quizartinib and other drug candidates and for working capital, capital expenditures and general

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corporate purposes. 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 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.

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 cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail 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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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words may, will, could, would should, expect, intend, plan, anticipate, believe, estimate, predict, project, potential, continue, ongoing or the negati similar expressions are intended to identify forward-looking 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;
- our ability to obtain and maintain regulatory approval of our product candidates, including quizartinib, and the labeling under any approval we may obtain;
- our plans to develop and commercialize our product candidates, including quizartinib;
- the loss of key scientific or management personnel;
- the size and growth of the potential markets for our product candidates and our ability to serve those markets;
- regulatory developments in the United States and foreign countries;
- the rate and degree of market acceptance of any future products;
- our use of the proceeds from this offering;
- the accuracy of our estimates regarding expenses, future revenues and capital requirements;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- the successful development of our sales and marketing capabilities;
- the success of competing drugs that are or become available; and
- the performance of third-party manufacturers.

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. We have included important factors in the cautionary statements included in this prospectus, particularly in the Risk Factors section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

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.

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 shares of common stock in this offering will be approximately $ million, assuming an initial public offering price of $ per share (the midpoint of the price range listed on the cover page of this prospectus) and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) our net proceeds from this offering by approximately $ 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 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 will be approximately $ million, assuming an initial public offering price of $ per share (the midpoint of the price range listed on the cover page of this prospectus) and after deducting 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 as follows:

- approximately $45.2 million to fund the continued clinical development of quizartinib and to begin building a U.S. sales force for quizartinib;
- approximately $16.0 million to fund the clinical development costs for AC480 IV and AC430; and
- the remainder for working capital and other general corporate purposes.

We believe that the net proceeds from this offering 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 approximately $61.2 million of the net proceeds from this offering intended for research and development and our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our clinical development efforts through the following events:

- submission of our NDA for quizartinib to the FDA;
- start of the confirmatory Phase 3 clinical trial for quizartinib;
- completion of a Phase 1 clinical trial of AC480 IV in combination with docetaxel; and
- completion of a Phase 1 clinical trial of AC430 in healthy volunteers.

The expected use of the net proceeds from this offering 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 product 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.

Pending use of the proceeds from this offering, we intend to invest the proceeds in a variety of capital preservation investments, including 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. 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, current portion of debt and capitalization as of September 30, 2010 (unaudited):

on an actual basis;

on an a pro forma basis to give effect to:

a -for- reverse stock split of our common stock to be effected prior to the closing of this offering;

the filing of our restated certificate of incorporation and the adoption of our restated bylaws as of the closing date of this offering;

no exercise by the underwriters of their option to purchase shares of common stock to cover over-allotments;

the issuance by us of 1,538,461 shares of our Series C-2 redeemable convertible preferred stock and 612,649 shares of Series D redeemable convertible preferred stock prior to the closing of this offering upon exercise of a put right held by GrowthWorks Canadian Fund Ltd., or the GrowthWorks put right;

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 24,608,183 shares of common stock upon the closing of this offering; and

the adjustment of outstanding warrants to purchase shares of our convertible preferred stock into warrants to purchase 649,573 shares of common stock upon the closing of this offering.

the issuance of shares of our common stock upon the closing of this offering as a result of the automatic conversion and/or cancellation of $15.0 million of secured notes (and accrued interest thereon) that we and Ambit Canada issued in September 2010 (including the shares issuable upon the automatic exercise of a warrant that we issued in September 2010 in connection with the issuance by Ambit Canada of a portion of the secured notes), assuming an initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) and assuming the conversion and/or cancellation occurs on , 2011 (for purposes of calculating the accrued interest on the notes).

on a pro forma as adjusted basis to additionally give effect to the sale of shares of common stock in this offering, assuming an initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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Our cash, current portion of debt 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 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 September 30, 2010		
	Actual	Pro Forma	Pro Forma as Adjusted[1]
	(unaudited, in thousands except share and per share data)		
Cash and cash equivalents	$ 37,318	$ 37,318	$
Current portion of notes payable, net of debt discount	$ 2,032	$ 2,032	$
Capitalization:			
Notes payable, net of current portion	$ 22,112	$	
Derivative liability- conversion feature	885		
Redeemable convertible preferred stock warrant liabilities	1,513		
Redeemable non-controlling interest	9,041		
Series A convertible preferred stock, $0.001 par value: 162,519 shares authorized; 46,666 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	445		
Series B convertible preferred stock, $0.001 par value: 1,975,677 shares authorized; 1,549,128 shares issued and outstanding actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	13,307		
Series C redeemable convertible preferred stock, $0.001 par value: 7,076,718 shares authorized, 5,139,734 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	21,899		
Series C-2 redeemable convertible preferred stock, $0.001 par value: 1,538,462 shares authorized; no shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted			
Series D redeemable convertible preferred stock, $0.001 par value: 21,000,000 shares authorized; 15,721,545 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	74,589		
Preferred stock, $0.001 par value: no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized, no shares issued and outstanding, pro forma and pro forma as adjusted			
Common stock, $0.001 par value: 44,800,000 shares authorized, 3,256,113 shares issued and outstanding, actual; 200,000,000 shares authorized, shares issued and outstanding, pro forma; 200,000,000 shares authorized and shares issued and outstanding, pro forma as adjusted	3		
Additional paid-in capital	23,579		
Accumulated other comprehensive income	210	210	
Accumulated deficit	(167,099)	(167,099)	
Total stockholders deficit	(143,307)		
Total capitalization	$ 484	$ 484	$

(1)

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share (which is the midpoint of the price range listed on the cover page of this prospectus) would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents and marketable securities, additional paid-in capital, total stockholders deficit and total capitalization by approximately $ 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 underwriting discounts and commissions and estimated offering expenses payable by us.

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The number of shares of our common stock to be outstanding after this offering is based on 27,864,296 shares of common stock outstanding as of September 30, 2010 and excludes:

- 5,452,559 shares of common stock issuable upon exercise of stock options outstanding as of September 30, 2010 at a weighted-average exercise price of $1.10 per share;

- 649,573 shares of convertible preferred stock issuable upon the exercise of warrants outstanding as of September 30, 2010 at a weighted-average exercise price of $4.64 per share (such warrants will be adjusted into warrants to purchase 649,573 shares of common stock upon the consummation of this offering);

- 2,539,590 shares of common stock issuable upon the exercise of warrants outstanding as of September 30, 2010 at a weighted-average exercise price of $1.06 per share; and

- an aggregate of 839,773 shares of common stock reserved for future issuance under our 2011 amended and restated equity incentive plan (referred to herein as our 2011 pre-IPO plan) as of September 30, 2010 and an aggregate of additional shares of common stock that will be available under our 2011 equity incentive plan (referred to herein as our 2011 post-IPO plan) and our 2011 employee stock purchase plan upon the closing of this offering.

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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 September 30, 2010 was approximately $143.3 million, or approximately $44.01 per share, based on the number of shares of common stock outstanding as of September 30, 2010. Historical net tangible book deficit per share is determined by dividing the number of shares of common stock outstanding as of September 30, 2010 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 24,608,183 shares of common stock, including the shares issued upon exercise of the GrowthWorks put right, 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, the reclassification of our redeemable convertible preferred stock warrant liabilities to additional paid-in capital, the conversion and/or cancellation of the 2010 bridge loans and related accrued interest through the assumed conversion date of , 2011 (including the shares issuable upon the exercise of a warrant that we issued in connection with the issuance by Ambit Canada of a portion of the 2010 bridge loans) into shares and the resulting reclassification of these liabilities and the related derivative liability-conversion feature to additional paid-in capital, our net tangible book deficit as of September 30, 2010 would have been approximately $ million, or approximately $ per share.

Investors participating in this offering will incur immediate, substantial dilution. After giving effect to the sale of common stock offered by us in this offering at an assumed initial public offering price of $ per share (the midpoint of the price range set forth on the cover page of this prospectus) net of underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of September 30, 2010 would have been approximately $ million, or approximately $ per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of $ per share to existing stockholders, and an immediate dilution of $ per share to investors participating in this offering. The following table illustrates this per share dilution:

Assumed initial public offering price per share		$
Historical net tangible book deficit per share as of September 30, 2010 (unaudited)	$ (44.01)	
Pro forma decrease in net tangible book deficit per share attributable to pro forma transactions described in preceeding paragraphs		
Pro forma net tangible book value (deficit) per share as of September 30, 2010 (unaudited)		
Pro forma increase in net tangible book value per share attributable to investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Pro forma dilution per share to investors participating in this offering		$

A $1.00 increase (decrease) in the assumed initial public offering price of $ 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 September 30, 2010 by approximately $ million, the pro forma as adjusted net tangible book value per share after this offering by $ and the dilution in pro forma as adjusted net tangible book value to new investors in this offering by $ 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 underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their over-allotment option in full to purchase additional shares of common stock in this offering, the pro forma as adjusted net tangible book value per share after the offering would be $ per share, the increase in the pro forma net tangible book value per share to existing

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stockholders would be $ per share and the dilution to new investors purchasing common stock in this offering would be $ per share.

The following table summarizes, on a pro forma as adjusted basis as of September 30, 2010, the differences between the number of shares of common stock purchased from us, the total consideration and the average price per share paid to us by existing stockholders and by investors participating in this offering, before deducting underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of $ 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		%	$	%	$
Investors participating in this offering					
Total		100%	$	100%	$

A $1.00 increase (decrease) in the assumed initial public offering price of $ 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 $ 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 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 % 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 , or % of the total number of shares of common stock to be outstanding after this offering.

The number of shares of our common stock outstanding as of September 30, 2010 was 27,864,296 shares and excludes:

- 5,452,559 shares of common stock issuable upon the exercise of outstanding options under our 2011 pre-IPO plan as of September 30, 2010 having a weighted-average exercise price of $1.10 per share;
- 649,573 shares of convertible preferred stock issuable upon the exercise of warrants outstanding as of September 30, 2010 at a weighted-average exercise price of $4.64 per share (such warrants will be adjusted into warrants to purchase 649,573 shares of common stock upon the consummation of this offering);
- 2,539,590 shares of common stock issuable upon the exercise of outstanding warrants having a weighted-average exercise price of $1.06 per share; and
- an aggregate of 839,773 shares of common stock reserved for future issuance under our 2011 pre-IPO plan as of September 30, 2010 and an aggregate of additional shares of common stock that will be available under our 2011 post-IPO plan and our 2011 employee stock purchase plan upon the closing of this offering.

Effective immediately upon the signing of the underwriting agreement for this offering, an aggregate of shares of our common stock will be reserved for issuance under our 2011 post-IPO plan and 2011 employee stock purchase plan, respectively, which includes shares of common stock reserved for future issuance under our 2011 pre-IPO plan that will be allocated to our 2011 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 financial statements and accompanying notes and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. The selected financial data in this section are not intended to replace our financial statements and the related notes. Our historical results are not necessarily indicative of our future results.

The selected statement of operations data for the years ended December 31, 2007, 2008 and 2009 and the selected balance sheet data as of December 31, 2008 and 2009 are derived from our audited financial statements appearing elsewhere in this prospectus. The selected statement of operations data for the years ended December 31, 2005 and 2006 and the selected balance sheet data as of December 31, 2005, 2006 and 2007 are derived from our audited financial statements which are not included in this prospectus. The selected statement of operations data for the nine months ended September 30, 2009 and 2010 and the selected balance sheet data as of September 30, 2010 are derived from our unaudited financial statements appearing elsewhere in this prospectus. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements included in this prospectus and include, in our opinion, all adjustments, consisting of normal recurring adjustments necessary for the fair presentation of the financial information in those statements.

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Statement of Operations Data:

	Years Ended December 31,					Nine Months Ended September 30,	
	2005	2006	2007	2008	2009	2009	2010
						(unaudited)	
	(in thousands except share and per share data)						
Revenues:							
Collaboration arrangements	$	$ 604	$ 3,621	$ 3,621	$ 3,466	$ 2,716	$ 14,782
Kinase profiling services (held-for-sale)	3,481	7,401	10,692	24,480	14,647	10,677	5,229
Total revenues	3,481	8,005	14,313	28,101	18,113	13,393	20,011
Operating expenses:							
Research and development	12,022	15,061	19,386	26,884	29,280	20,371	29,155
General and administrative	4,259	4,438	6,466	6,581	5,788	4,134	6,294
In-process research and development			25,000				
Cost of kinase profiling services revenue (held-for-sale)		2,658	2,993	4,194	3,777	2,888	1,298
Total operating expenses	16,281	22,157	53,845	37,659	38,845	27,393	36,747
Loss from operations	(12,800)	(14,152)	(39,532)	(9,558)	(20,732)	(14,000)	(16,736)
Other income (expense):							
Interest expense	(400)	(1,717)	(1,874)	(1,736)	(4,899)	(2,319)	(9,676)
Other income (expense)	521	705	946	1,202	(364)	(278)	(7)
Change in fair value of redeemable convertible preferred stock warrant liabilities		297	278	258	(658)	(243)	337
Total other income (expense)	121	(715)	(650)	(276)	(5,921)	(2,840)	(9,346)
Loss before income taxes	(12,679)	(14,867)	(40,182)	(9,834)	(26,653)	(16,840)	(26,082)
Provision for (benefit from) income taxes		38	196		(191)		1,900
Consolidated net loss	(12,679)	(14,905)	(40,378)	(9,834)	(26,462)	(16,840)	(27,982)
Net loss attributable to redeemable non-controlling interest	49	260	411	86	2,177	1,245	1,446
Net loss attributable to Ambit Biosciences Corporation	(12,630)	(14,645)	(39,967)	(9,748)	(24,285)	(15,595)	(26,536)
Accretion to redemption value of redeemable convertible preferred stock	(4,359)	(4,627)	(3,867)	(61)	(61)	(46)	(626)
Change in fair value of redeemable non-controlling interest	1,615	1,602	(180)	1,737	(7,567)	(3,384)	702
Net loss attributable to common stockholders	$ (15,374)	$ (17,670)	$ (44,014)	$ (8,072)	$ (31,913)	$ (19,025)	$ (26,460)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (17.20)	$ (19.64)	$ (47.30)	$ (8.38)	$ (15.47)	$ (11.39)	$ (8.15)
Weighted-average shares outstanding, basic and diluted[1]	893,848	899,825	930,465	963,390	2,063,489	1,671,012	3,247,170

Pro forma net loss per share, basic and diluted (unaudited)[1]	$	(1.37)	$
Weighted-average pro forma shares outstanding, basic and diluted (unaudited)[1]		18,828,136	

(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 and the number of shares used in computation of the per share amounts.

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Balance Sheet Data:

	As of December 31,					As of September 30,
	2005	2006	2007	2008	2009	2010 (unaudited)
	(in thousands)					
Cash, cash equivalents and short-term investments	$ 18,631	$ 23,141	$ 55,392	$ 15,364	$ 40,798	$ 37,318
Working capital (deficit)	15,580	9,930	14,505	(4,240)	26,712	26,276
Total assets	25,501	31,997	64,366	26,169	48,762	48,086
Total notes payable	7,069	13,856	13,547	8,320	25,868	24,144
Redeemable convertible preferred stock	35,963	40,590	75,635	75,696	67,081	96,488
Convertible preferred stock	18,283	18,283	18,283	18,283	13,752	13,752
Accumulated deficit	(49,438)	(66,563)	(106,530)	(116,278)	(140,563)	(167,099)
Total stockholders deficit	(48,953)	(66,426)	(94,851)	(104,289)	(120,838)	(143,307)

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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 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 biotechnology company engaged in discovering, developing and commercializing targeted small molecule therapeutics for the treatment of cancer. Our drug candidates are directed against an important family of enzymes called kinases, known to be involved in a range of human diseases. We are developing our lead drug candidate, quizartinib (formerly AC220), for the treatment of acute myeloid leukemia, or AML, under our global collaboration with Astellas Pharma Inc. and Astellas US LLC, collectively Astellas. Quizartinib is a once-daily, orally-administered, potent and selective kinase inhibitor currently in a pivotal Phase 2 clinical trial as monotherapy in relapsed/refractory AML. Quizartinib is being developed in concert with a companion diagnostic test to identify and treat the approximately one-third of AML patients with activating mutations in the FLT3 gene that drive a particularly aggressive and deadly form of this disease. We believe a targeted and personalized medicine approach to the treatment of AML has significant potential to improve patient outcomes and may transform what is an aggressive and deadly disease into a manageable condition. Novartis Gleevec (imatinib), a targeted kinase inhibitor, accomplished a similar transformation in the treatment of chronic myeloid leukemia. In addition to quizartinib, we have a pipeline of kinase inhibitors aimed at addressing significant unmet medical needs with potential advantages over existing therapeutics.

In December 2009, we entered into a worldwide agreement with Astellas to jointly research, develop and commercialize FLT3 kinase inhibitors. As partial consideration for the exclusive license rights granted to Astellas, we received an upfront payment of $40.0 million. In addition, we may receive payments of up to $350.0 million upon the achievement of development and regulatory milestones. We are also entitled to receive tiered double-digit royalty payments on sales as well as annual sales-based milestones. The agreement provides that we and Astellas will conduct a joint five-year research program related to certain designated follow-on compounds to quizartinib. We and Astellas share development costs in the United States and European Union and the research costs on follow-on compounds equally. Astellas is responsible for all other development costs and the costs associated with commercialization of products covered by the agreement. We retain the right to co-promote quizartinib and any follow-on drugs in the United States, in which case we and Astellas will share equally any commercialization costs in the United States.

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 product 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, or DiscoveRx, pursuant to an asset purchase agreement. In consideration for the sale of such assets, DiscoveRx paid us $7.3 million at the closing of the transaction and may be required to pay us up to an additional $4.9 million upon the achievement of certain sales and operational milestones. In the event of certain changes of control of DiscoveRx prior to December 31, 2012, up to $4.5 million of any unpaid milestones could become immediately due and payable to us. We are obligated to purchase from DiscoveRx a minimum of $0.6 million of screening services during each full calendar quarter through December 31, 2012, with the first quarter through December 31, 2010 being prorated from the close date through the end of the quarter. As a result of the commitment to purchase minimum levels of screening services, we will initially defer $5.5 million of the gain on the sale transaction. To the extent minimum quarterly commitments exceed the actual

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amount of services received, we will have to pay the difference, which will be accounted for as a reduction in both the sales price and the overall gain to be recorded on the sale of the business. As part of the asset purchase agreement we have 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 their sale to DiscoveRx. We have agreed with DiscoveRx only to grant sublicenses to such intellectual property rights to third parties that have agreed to conduct research and development programs regarding products to which we have substantial rights and/or material interests other than royalties, or which result from our internal development efforts. We have further agreed that we will not utilize such licensed intellectual property rights other than in connection with such research and development programs.

To date, 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 associated with our collaboration agreements and from our former kinase profiling services business. We have incurred consolidated net losses of approximately $40.4 million, $9.8 million and $26.5 million in the years ended December 31, 2007, 2008 and 2009, respectively, and approximately $16.8 million and $28.0 million during the nine months ended September 30, 2009 and 2010 respectively. As of September 30, 2010 we had an accumulated deficit of approximately $167.1 million.

We expect to incur significant and increasing operating losses for the foreseeable future as we advance our product candidates from discovery through preclinical studies and clinical trials, seek regulatory approval and pursue eventual commercialization. We will need additional financing to support our operating activities. We will seek to fund our operations through public or private equity or debt financings or other sources, such as strategic partnerships. Adequate additional funding may not be available to us on acceptable terms, or at all. We expect that research and development expenses will increase along with general and administrative costs, as we grow and operate as a public company. We will need to generate significant revenues to achieve profitability and we may never do so.

We conduct the majority of our activities through our parent company, Ambit Biosciences Corporation, from our primary facility in San Diego, California. Additionally, we own 50% of Ambit Biosciences (Canada) Corporation, or Ambit Canada, which conducts some of our research and development activities in Toronto. As discussed further in Note 2 to our consolidated financial statements, Ambit Canada is consolidated for financial reporting purposes.

The following information is presented on a consolidated basis to include the accounts of us, our wholly owned subsidiary Ambit Europe Limited (Ambit Europe), located in the United Kingdom, and our controlled subsidiary, Ambit Canada, each of which have limited operations. All intercompany transactions and balances are eliminated in consolidation.

Financial Overview

Revenues

To date, we have not generated any revenues from product sales. We have generated revenues from two primary sources: (i) payments from collaboration arrangements and (ii) kinase profiling services fees through October 2010, at which time the service business was sold. Collaboration arrangements typically include payment to us of one or more of the following: nonrefundable, upfront license fees; milestone payments; sponsored research payments (fees for research and development services rendered); and royalty payments on product sales. 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 product candidates unless and until they obtain regulatory approval. To date, we have not submitted any drug candidate for regulatory approval. Kinase profiling services relate to our former use of our panel of kinase assays for third parties.

In the future, we may generate revenues from a combination of product sales, license fees, milestone payments and research and development payments and royalties in connection with strategic partnerships. We expect that any revenues we generate will fluctuate from quarter to quarter as a result of the timing and amount

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of license fees, research and development reimbursements, milestone and other payments received under our strategic partnerships, and the payments that we receive upon the sale of our products, to the extent any are approved and successfully commercialized. If we or our strategic partners fail to complete the development of our drug candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenues, and our results of operations and financial position, would be materially adversely affected.

Additionally, we do not expect future revenues from kinase profiling services as we sold the service portion of our business in October 2010 to focus on drug discovery and development.

Research and Development

Research and development expenses relate to the discovery and development of our product candidates. Our business model is dependent upon our continuing to conduct a significant amount of research and development. Product candidates in late stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of late stage clinical trials. We plan to increase our research and development expenses for the foreseeable future as we seek to complete development of our most advanced product candidate, quizartinib, and to further advance the earlier-stage research and development programs in our pipeline. Quizartinib represents the largest portion of our research and development expense. Under our agreement with Astellas, we share quizartinib development costs in the United States and European Union and research costs on follow-on compounds equally with Astellas. Astellas is responsible for all other development costs. 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 acquiring and manufacturing 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:

	Years Ended December 31,			Nine Months Ended September 30,		Total January 1, 2007 through September 30, 2010
	2007	2008	2009	2009 (unaudited)	2010 (unaudited)	
			(in thousands)			
Quizartinib	$ 2,669	$ 5,493	$ 12,276	$ 7,523	$ 15,151	$ 35,589
AC430	1,562	2,613	2,361	1,298	3,148	9,684
Discovery projects	8,515	9,798	5,954	4,391	4,823	29,090

Kinase profiling services	4,388	3,096	3,150	2,399	2,641	13,275
AC480	1,744	3,968	3,565	2,782	1,703	10,980
R&D administration	508	1,916	1,974	1,978	1,689	6,087
Total	$ 19,386	$ 26,884	$ 29,280	$ 20,371	$ 29,155	$ 104,705

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

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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 and our preclinical program, our future research and development expenses will depend on the preclinical and clinical success of each product candidate that we develop, as well as ongoing assessments of the commercial potential of such product candidates. In addition, we cannot forecast with any degree of certainty which product 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.

We expect to incur increased research and development expenses as we continue to enroll patients in our current pivotal Phase 2 clinical trial of quizartinib as monotherapy in relapsed/refractory AML patients with and without internal tandem duplication, or ITD, mutations in the FLT3 gene, which we refer to as FLT3-ITD positive patients. In addition, we expect to incur significant research and development costs as we initiate future trials including a confirmatory Phase 3 clinical trial of quizartinib in FLT3-ITD positive patients, expected to begin in the second half of 2011. Research and development expenditures will continue to increase as we advance the development of our proprietary pipeline of novel drug candidates, including AC480 and AC430.

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

- per patient trial costs;
- 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 patients that participate in the trials;
- 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 product candidate.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for personnel in executive, finance, business development, information technology, marketing, and legal functions. Other general and administrative expenses include facility costs (not otherwise included in cost of kinase profiling services or research and development expenses), patent filing costs, and professional fees for legal, consulting, auditing and tax services.

We anticipate that our general and administrative expenses will increase for, among others, the following reasons:

- as a result of increased payroll, expanded infrastructure and higher consulting, legal, accounting and investor relations costs, and director and officer insurance premiums associated with being a public company;
- to support our research and development activities, which we expect to expand as we continue the development of our product candidates; and
- to build a sales and marketing team before we receive regulatory approval of a product candidate in anticipation of commercial launch.

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In-Process Research and Development

In October 2007, we and Bristol-Myers Squibb Company, or BMS, entered into: (i) a license agreement pursuant to which we acquired an exclusive license to certain patents and other intellectual property related to AC480, and (ii) a licensing and profiling services agreement. Under the terms of the agreements, we received a $6.0 million upfront payment and received the worldwide product rights to AC480. In exchange for the cash payment and the worldwide product rights to AC480, we were obligated to provide kinase profiling services to BMS for which we would not receive additional compensation. This obligation was fulfilled in the first half of 2010.

We recorded the receipt of the worldwide product rights to AC480 based on its fair value. The fair value of the AC480 compound was determined utilizing the market approach, assuming that the fair value of the AC480 compound rights can be determined by a review of available valuations of identified comparable compounds to approximate the value of the AC480 compound. The market approach makes use of publicly available information on assets that are deemed to be similar to the AC480 compound. In selecting comparable compounds, we targeted then-approved kinase inhibitors or kinase inhibitors under clinical development as comparable compounds to AC480. After selecting comparable compounds, a review of license agreements involving the comparable compounds was conducted. For purposes of the application of this method, only upfront cash payments and committed cash R&D support were used to determine the implied value of the comparables. Milestone, royalty, or profit splits were excluded from the fair value calculation due to the early nature of these compounds and the uncertainty regarding the timing and achievability of any milestone, royalty, or profit split terms. Under the methodology described above, we identified four comparable Phase 1 cancer licensing deals with R&D support payments and estimated a fair value of $25.0 million for the AC480 compound. Because the acquired AC480 compound is in the early stage of the development cycle, the in-process R&D project was expensed immediately upon receipt from BMS.

Cost of Kinase Profiling Services (held-for-sale)

Cost of kinase profiling services represents expenses associated with the delivery of kinase profiling services to third parties. Cost of kinase profiling services consists primarily of raw materials, compensation, benefits and other employee related expenses, as well as an allocation of facility costs. We do not expect these costs to continue in the future as we sold the kinase profiling service portion of our business in October 2010 to focus on drug discovery and development.

Interest Expense

Interest expense consists primarily of coupon interest, amortization of debt discount, beneficial conversion charges, accretion to principal repayment premiums and amortization of deferred financing costs associated with our loans payable.

Other Income (Expense)

Other income (expense) consists primarily of: (i) interest income earned on our cash and cash equivalents and marketable securities and (ii) exchange rate gains and losses on transactions denominated in a currency other than the functional currency.

Change in Fair Value of Redeemable Convertible Preferred Stock Warrant Liabilities

For redeemable convertible preferred stock warrants and put instruments that are accounted for as liabilities, the value of such instruments is re-measured at each financial reporting period. As the value of such instruments is primarily related to the fair value of our stock, in periods where the underlying stock value has gone up, a non-operating expense is recorded. Conversely, if our stock price declines, the decrease in the liability results in non-operating other income being recorded.

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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. 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 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 from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 1 to our 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.

Revenue Recognition

Our revenues generally consist of: (i) payments from collaboration arrangements and (ii) kinase profiling services fees through October 21, 2010, at which point the kinase profiling service business was sold. 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 revenue type is outlined below.

Collaboration Arrangements

We have entered into various collaboration arrangements, including those with Astellas, BMS and Cephalon, Inc., which contain multiple elements. A variety of factors are considered in determining the appropriate method of revenue recognition under these arrangements, such as whether the various elements can be considered separate units of accounting, whether there is objective and reliable evidence of fair value for these elements and whether there is a separate earnings process associated with a particular element of an agreement.

Where there are multiple deliverables that do not have stand-alone value to the collaborator, the non-contingent consideration from these deliverables are combined into separate units of accounting based on their relative fair values. Applicable revenue recognition criteria are considered separately for each unit of accounting. Non-contingent revenues from the combined unit of accounting are deferred and recognized over the period that we remain obligated to perform services or deliver product. The specific methodology for the recognition of the revenues (e.g., straight-line or according to specific performance criteria) is determined on a case-by-case basis according to the facts and circumstances applicable to a given contract.

Specifically, the revenue recognition methodology for the various elements in our multiple element arrangements is as follows:

Upfront licensing fees. The Company recognizes revenues from nonrefundable, upfront license fees for which the separation criteria were not met, due to continuing involvement in the performance of research and development services on a straight-line basis over the contracted or estimated period of performance, which is typically the research or development term.

Milestones. Milestone payments are derived from the achievement of predetermined events under collaboration arrangements and are assessed on an individual basis. Revenues are not recognized for milestones that are subject to contingencies until the revenues are earned, as evidenced by acknowledgment from the collaborator, provided that: (i) the milestone event is substantive and its achievability was not reasonably assured at the inception of the agreement, (ii) the milestone represents the culmination, or progress towards the

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culmination, of an earnings process and (iii) the milestone payment is non-refundable. Where separate milestones do not meet these criteria, we default to a performance-based model, with revenue recognition following delivery of effort as compared to an estimate of total expected effort. Milestones that are received after all substantive deliverables have occurred are considered to be bonus payments and are recognized upon receipt of the cash, assuming all of the other revenue recognition criteria are met.

Collaborative research payments. Collaborative research payments are primarily based on: (i) time worked using a contractual cost per full-time equivalent employee working on the project and (ii) direct costs associated with the project. We recognize revenues related to these payments as the services are performed and costs are incurred over the related funding periods for each agreement, assuming all other revenue recognition criteria have been met. Payments received in excess of revenue recognized are recorded as deferred revenues until: (i) sufficient time billable to the project has been incurred and/or (ii) related project costs have been expended.

Collaboration arrangements also include potential payments for product royalty, commercial product supply, and sharing of operating profits. To date, we have not received payments or recorded revenues from any of these sources.

Kinase Profiling Services (held-for-sale)

Kinase profiling services were provided on a fee-for-service basis through October 21, 2010, the date we sold this business, and were billed when the profiling results data was provided to the customers. We recognized revenues upon delivery of the profiling data to the customer, assuming all other revenue recognition criteria have been met. Amounts received in advance of services performed were recorded as deferred revenues until earned.

Accrued Clinical Expenses

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

Examples of estimated accrued clinical expenses include:

- fees paid to CROs in connection with clinical trials;
- fees paid to investigative sites in connection with clinical trials;
- fees paid to contract manufacturers in connection with the production of clinical trial materials; and
- fees paid to vendors in connection with the preclinical development activities.

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

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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-valuation model. The Black-Scholes model requires the input of subjective assumptions, including the expected stock price, volatility, the calculation of 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:

	Years Ended December 31,			Nine Months Ended September 30,	
	2007	2008	2009	2009	2010
Risk-free interest rate	4.5%	3.0%	2.3%	2.2%	2.0%
Expected dividend yield					
Expected volatility	63.2%	59.5%	61.0%	61.0%	62.2%
Forfeiture rate	14.8%	13.7%	12.2%	12.2%	12.2%
Expected term of options (years)	6.1	6.2	6.1	6.1	6.1

Risk-free Interest Rate. The risk-free interest rate assumption was based on zero coupon United States Treasury instruments that had terms consistent with the expected term of our stock option grants.

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

Expected Volatility. The expected volatility rate used to value stock option grants is 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 biotechnology industry in a similar stage of development.

Forfeiture Rate. We estimate forfeitures based on historical experience at the time of grant and revise our estimate, if necessary, in subsequent periods if actual forfeitures differ from such estimates.

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 September 30, 2010, due to the absence of an active market for our common stock, the exercise prices for all options granted were at the estimated fair value as determined contemporaneously on the date of grant by our board of directors prepared in accordance with methodologies outlined in the AICPA Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Our board of directors, which includes members who are experienced in valuing the securities of biotechnology/pharmaceutical companies, considered a number of subjective and objective factors including:

- the prices of our convertible preferred stock sold to outside investors in arms-length transactions, and the rights, preferences and privileges of our convertible preferred stock as compared to those of our common stock, including the conversion rights and liquidation preferences of our convertible preferred stock;

- our results of operations, financial position and the status of our research and development efforts, including results from our clinical trials;

our stage of development and business strategy;

the composition of and changes to our management team;

the market value of a comparison group of publicly-held pharmaceutical and biotechnology companies that are in a stage of development similar to ours;

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- the lack of liquidity of our common stock as a private company;
- contemporaneous valuation data provided by management;
- the likelihood of achieving a liquidity event for the shares of our common stock and underlying stock options, such as an initial public offering, given prevailing market conditions; and
- the material risks related to our business.

Based on these factors, our board of directors granted options at exercise prices that have ranged from a low of $0.50 per share in 2004 up to a high of $1.54 per share in November 2010.

In connection with the preparation of the 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 2009 and 2010. The reassessment included both the determination of the appropriate valuation model and related inputs. For grants made between January 1, 2009 and February 4, 2009, we concluded that the reassessed fair value of common stock was lower than the exercise price of options granted. For option grants made February 5, 2009 through September 30, 2010, we concluded that the reassessed fair value of common stock was higher than the exercise price of options granted. We used these fair value reassessments to determine stock-based compensation expense which is recorded in our financial statements.

Our reassessment analysis was 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 three primary factors: (i) a market approach using publicly traded comparables, (ii) a market approach using mergers and acquisitions, or M&A, transaction comparables, and (iii) an income approach using discounted cash flow analysis. The market approach using publicly traded comparables is based on revenue multiples derived from already public companies that are focused on oncology and have other similar characteristics, including size and business model. The market approach using M&A transaction comparables is based on revenue and other multiples derived from M&A transactions for companies in the oncological pharmaceutical industry. The income approach using a discounted cash flow analysis is based on the residual value and free cash flow from our multi-year forecast discounted to present value based on our calculated weighted-average cost of capital.

For 2009 and the first two quarters of 2010, once our enterprise value was determined under each method, we adjusted for our interest bearing debt, and then allocated such value to our different classes of equity using the option pricing method. The option pricing method utilizes the conversion rights and liquidation preferences of each class of stock and the Black-Scholes options pricing model to calculate the fair value of each class of stock based on each security s relative right to our enterprise value, as adjusted for outstanding interest bearing debt, and the lack of marketability of our common stock. We selected the option pricing method to allocate our enterprise value from several alternative methods, including the probability weighted expected return method, or PWERM, due to our lack of clarity as to the timing and form of a potential liquidity event at the time the reassessed valuations were prepared. These analyses were performed prior to receiving the results from our pivotal Phase 2 clinical trial for quizartinib. Since the option pricing method utilizes conversion rights and liquidation preferences to allocate our enterprise value, such methodology allocates a large portion of our enterprise value to our convertible preferred stock. The disparity in preferred and common stock values is reflective of the significant development risks outstanding at the date of each reassessment.

During the third quarter of 2010, we initiated our initial public offering process. As a result, we selected the PWERM to allocate value since we believed we had greater clarity as to the timing and form of potential liquidity events. The PWERM considers the present value of the returns afforded to stockholders under each of four possible future scenarios. These scenarios consisted of: (i) an initial public offering in January 2011, (ii) a sale in June 2011, (iii) an initial public offering in September 2011 and (iv) a liquidation in December 2011. The following probabilities were assigned: (i) a 74% combined probability assigned to the two initial public offering scenarios, (ii) a 25% probability assigned to the sale scenario and (iii) a 1% probability assigned to the liquidation scenario. Under the initial public offering scenarios, value was allocated on a fully diluted basis, while the sale scenario took into account the conversion rights and liquidation preferences of each class of stock. Under both scenarios the option and warrant holders were assumed to exercise to the extent the exercise prices of

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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 and our 37% weighted-average cost of capital, probability weighted as discussed above and discounted by 10% to adjust for lack of marketability.

The following table summarizes the values of our common stock on a quarterly basis:

	Reassessed Value
March 31, 2009	$ 0.61
June 30, 2009	0.81
September 30, 2009	0.96
December 31, 2009	1.54
March 31, 2010 (unaudited)	1.45
June 30, 2010 (unaudited)	1.62
September 30, 2010 (unaudited)	2.98

The aggregate $0.35 per share increase in the fair value of our common stock during the second and third quarters of 2009 is representative of modest increases in our overall enterprise value as we advanced our business model, particularly the progression towards our obtaining FDA clearance to initiate our pivotal Phase 2 clinical trial for quizartinib. Within the second quarter of 2009, we did not reflect any increase in valuation until June when we received additional financing from our investors. Prior to that time, we had significant liquidity risk as we continued to operate with very limited cash reserves. There were no identifiable milestones or events from an operating perspective that would have offset the increasing liquidity risk and increased overall intrinsic value. The $0.58 per share increase in the fair value of our common stock between September 30, 2009 and December 31, 2009 is primarily attributed to the increase in our enterprise value in connection with our global collaboration agreement with Astellas which was executed on December 18, 2009. The collaboration agreement resulted in a $40.0 million upfront cash payment and provides for cost sharing and potential milestone and royalty payments going forward. The $0.08 per share increase in the value of our common stock from December 31, 2009 to June 30, 2010 reflects an increase in our enterprise value consistent with our continued enrollment in our pivotal Phase 2 clinical trial, and the filing with the FDA of our IND for AC480 in April 2010. The $1.36 per share increase in the value of our common stock from June 30, 2010 to September 30, 2010 was heavily influenced by: (i) our engagement of investment bankers and related commencement of the initial public offering process, (ii) the hiring of a chief executive officer, (iii) receipt of interim data on our pivotal Phase 2 clinical trial and (iv) greater clarity as to potential liquidity events, in particular, the higher probability of an initial public offering. While our weighted enterprise value remained relatively constant, our common stock valuation increased significantly due to the assignment of a higher probability of an initial public offering occurring in early or late 2011 which results in a shift in the allocation between the preferred and common stockholders as the preferred stockholders were assumed to convert to common stock in the initial public offering scenarios, giving up their liquidation preferences.

Determining the fair market value of our common stock involves complex and subjective judgments including estimates of revenues, assumed market growth rates and estimated costs, as well as appropriate discount rates. At the time of each valuation, the significant estimates used in the discounted cash flow approach included estimates of our revenues and revenue growth rates for several years into the future. Although each time we prepared such forecasts for use in the preparation of a valuation report, we did so based on assumptions that we believed to be reasonable and appropriate, there can be no assurance that any such estimates for earlier periods or for future periods will prove to be accurate.

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Summary of Stock Option Grants

The following table compares the originally determined value (exercise price) and reassessed value:

Grant Date	Shares Subject to Options Granted	Exercise Price per Share	Reassessed Value of Common Stock per Share at Date of Grant	Intrinsic Value Per Share at Date of Grant
February 4, 2009	291,613	$ 0.91	$ 0.63	$
March 31, 2009	30,000	0.59	0.61	0.02
April 30, 2009	30,000	0.59	0.61	0.02
May 21, 2009	200,000	0.59	0.61	0.02
May 29, 2009	20,000	0.59	0.61	0.02
November 3, 2009	45,000	0.59	0.96	0.37
November 30, 2009	56,500	0.59	0.95	0.36
June 29, 2010 (unaudited)	507,500	1.54	1.62	0.08
August 19, 2010 (unaudited)	1,679,880	1.54	2.98	1.44
August 25, 2010 (unaudited)	385,500	1.54	2.98	1.44
September 30, 2010 (unaudited)	17,000	1.54	2.98	1.44

Subsequent to September 30, 2010, we granted options to purchase an aggregate of 575,200 shares of our common stock. Between September 30, 2010 and the grant dates of such options, we determined that (i) no additional corporate milestones had been achieved, (ii) no additional funding had occurred and (iii) there were no changes in our debt structure, any of which would have caused the fair value of our common stock to change. As such, the 575,200 options issued between October 1 and November 3, 2010, were issued at a fair value of $2.98 and an exercise price of $1.54. These options have a grant date fair value of approximately $1.2 million which will be expensed over the requisite service period. There have been no option grants subsequent to November 3, 2010.

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

	Year Ended December 31,			Nine Months Ended September 30,	
	2007	2008	2009	2009	2010
				(unaudited)	
			(in thousands)		
Research and development	$ 59	$ 128	$ 125	$ 87	$ 195
General and administrative	25	112	114	72	278
Total	$ 84	$ 240	$ 239	$ 159	$ 473

Total share-based compensation expense related to unvested stock option grants not yet recognized as of September 30, 2010 was approximately $4.7 million and the weighted-average period over which these grants are expected to vest is 3.7 years.

Based on the assumed initial public offering price of $ per share (the midpoint of the range set forth on the cover page of this preliminary prospectus), the intrinsic value of stock options outstanding as of September 30, 2010 would be $, of which $ and $ 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. We recorded share-based compensation for options granted to non-employees of approximately $4,000 and $15,000 for the years ended December 31, 2007 and 2008. No non-employee share-based compensation expense was recorded for the year ended December 31, 2009 or the nine month periods ended September 30, 2009 and 2010.

Common Stock Warrants

We have estimated the fair value of all outstanding common stock warrants as of the date of grant. Such values were estimated using the Black-Scholes option-pricing model and an estimated term equal to each warrant s contractual term of ten years. The value is included as a component of stockholders deficit.

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Redeemable Convertible Preferred Stock Warrant Liabilities

We have issued freestanding warrants to purchase shares of our redeemable convertible preferred stock. The redeemable convertible preferred stock warrants are exercisable for shares of Series C and Series D 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 redeemable convertible preferred stock warrants are recorded at fair value using either the Black-Scholes option pricing model or a binomial model. We used the Black-Scholes option pricing model to value all warrants except the warrants issued on March 31, 2010, which included anti-dilution terms that could change the settlement amount and therefore a binomial model was used to value these warrants. The anti-dilution terms of these warrants expire upon the closing of this offering when the warrants are converted to common stock warrants. The fair value of all redeemable convertible preferred stock warrants is re-measured at each financial reporting period with any changes in fair value being recognized in change in fair value of redeemable convertible preferred stock warrant liabilities, a component of other income (expense), in the accompanying consolidated statements of operations. We will continue to re-measure the fair value of the warrant liability 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 in connection with an initial public offering.

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 earlier of: (i) exercise of the underlying put rights or (ii) the time at which GrowthWorks no longer holds Class C and Class D shares in Ambit Canada, at which time the redeemable non-controlling interest will be reclassified to additional paid-in capital.

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 United States generally accepted accounting principles, 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 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 Nine Months Ended September 30, 2009 and 2010 (unaudited)

Revenues

The following table summarizes our revenues for the nine months ended September 30, 2009 and 2010:

	Nine Months Ended September 30,		
	2009	2010	Increase (Decrease)
	(in thousands)		
Collaboration arrangements	$ 2,716	$ 14,782	$ 12,066
Kinase profiling services (held-for-sale)	10,677	5,229	(5,448)
	$ 13,393	$ 20,011	$ 6,618

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Collaboration Arrangements Revenues. In December 2009, we entered into a collaboration arrangement with Astellas, which resulted in revenues during the nine-month period ended September 30, 2010 of approximately $13.8 million. In addition, we recognized a milestone payment of $1.0 million under our collaboration with Cephalon in February 2010. Revenues from collaboration arrangements in 2009 were primarily related to our 2006 agreement with Cephalon, which terminated in November 2009 resulting in no revenues from this agreement during the nine-month period ended September 30, 2010.

Kinase Profiling Services Revenues (held-for-sale). Services revenues decreased primarily due to decreases of $4.8 million and $0.7 million in screening activity for BMS and Cephalon, respectively, as obligations under these contracts were met. We do not expect future revenues from kinase profiling services as we sold the service portion of our business in October 2010.

Operating Expenses

The following table summarizes our operating expenses for the nine months ended September 30, 2009 and 2010:

	Nine Months Ended September 30,		
	2009	2010	Increase (Decrease)
	(in thousands)		
Research and development	$ 20,371	$ 29,155	$ 8,784
General and administrative	4,134	6,294	2,160
Cost of kinase profiling services (held-for-sale)	2,888	1,298	(1,590)
	$ 27,393	$ 36,747	$ 9,354

Research and Development Expense. Research and development expenses in the nine months ended September 30, 2009 and 2010, respectively, related primarily to the continued development of quizartinib and other preclinical programs. The increase in 2010 was primarily driven by: (i) a $7.6 million increase in quizartinib costs associated with clinical trial expenses, and (ii) a $1.9 million increase in AC430 costs associated with preclinical testing and a $0.4 million increase in discovery projects for potential commercialization. These increases were partially offset by a decrease of $1.1 million in AC480 costs due to increased focus on quizartinib. We expect research and development costs to increase as a result of the clinical trial program for quizartinib and the continued development of AC480, AC430 and our ongoing research activities.

General and Administrative Expense. General and administrative expenses increased primarily due to: (i) a severance charge of approximately $1.3 million associated with a former officer, (ii) $0.7 million in professional services, including completion of our 2009 financial audit and related costs to prepare for reporting as a public company, (iii) an increase of approximately $0.2 million associated with stock-based compensation. These increases were partially offset by a $0.1 million decrease in net salaries and related benefits associated with the former officer s departure.

Cost of Kinase Profiling Services Revenues (held-for-sale). Cost of services consists primarily of compensation, benefits and other employee related expenses, as well as an allocation of facility costs. The decrease in cost of profiling revenues was driven by a corresponding decrease in services revenues and related activities. We do not expect these costs to continue in the future as we sold the service portion of our business in October 2010.

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Other Income (Expense)

The following table summarizes our other income (expense) activity for the nine months ended September 30, 2009 and 2010:

	Nine Months Ended September 30, 2009	2010	Change
		(in thousands)	
Interest expense	$ (2,319)	$ (9,676)	$ (7,357)
Other expense	(278)	(7)	271
Change in fair value of redeemable convertible preferred stock warrant liabilities	(243)	337	580