AMBIT BIOSCIENCES CORP
Form 10-Q
August 13, 2013
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2013

OR

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

From the transition period from _____ to _____.

Commission File Number 001-35919

AMBIT BIOSCIENCES CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	**33-0909648**
(State or other jurisdiction of incorporation)	**(IRS Employer Identification No.)**
11080 Roselle St., San Diego, CA	**92121**
(Address of principal executive offices)	**(Zip Code)**

Registrant s telephone number, including area code: (858) 334-2100

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

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

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

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

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

As of July 31, 2013, there were 17,876,704 shares of common stock of the issuer outstanding.

Table of Contents

AMBIT BIOSCIENCES CORPORATIONS

FORM 10-Q

TABLE OF CONTENTS

Table of Contents

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

Ambit Biosciences Corporation

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

	June 30, 2013 (unaudited)	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$ 85,285	$ 17,481
Prepaid expenses and other current assets	935	1,231
Total current assets	86,220	18,712
Property and equipment, net	799	560
Deposits and other assets		717
Restricted cash	63	
Total assets	$ 87,082	$ 19,989
Liabilities and stockholders equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	$ 5,783	$ 7,290
Accrued payroll and related expenses	1,323	1,313
Current portion of notes payable, net of discount	1,798	4,320
Current portion of deferred revenue	6,947	6,362
Warrant liabilities	6,965	10,540
Total current liabilities	22,816	29,825
Deferred revenue, net of current portion		14,309
Redeemable non-controlling interest		3,323
Commitments and contingencies		
Convertible preferred stock, $0.001 par value:		
Authorized shares 10,000,000 and 170,990,763 at June 30, 2013 and December 31, 2012, respectively		
Redeemable convertible preferred stock:		
Issued and outstanding shares - 0 and 121,826,424 at June 30, 2013 and December 31, 2012, respectively; liquidation preference - $0 and $202,475 at June 30, 2013 and December 31, 2012, respectively		157,076
Convertible preferred stock;		
Issued and outstanding shares - 0 and 1,590,014 at June 30, 2013 and December 31, 2012, respectively; liquidation preference - $0 and $13,702 at June 30, 2013 and December 31, 2012, respectively		13,702
Stockholders equity (deficit):		
Common stock, $0.001 par value;		
Authorized shares - 200,000,000 and 225,000,000 at June 30, 2013 and December 31, 2012, respectively; issued and outstanding shares -17,876,704 and 3,990 at June 30, 2013 and December 31, 2012, respectively	18	

Additional paid-in capital	304,383	38,678
Accumulated other comprehensive (loss) income	(83)	47
Accumulated deficit	(240,052)	(236,971)
Total stockholders equity (deficit)	64,266	(198,246)
Total liabilities and stockholders equity (deficit)	$ 87,082	$ 19,989

See accompanying notes.

Table of Contents

Ambit Biosciences Corporation

Condensed Consolidated Statements of Comprehensive Income (Loss)

(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
	(unaudited)			
Revenues:				
Collaboration agreements	$ 11,547	$ 5,174	$ 18,139	$ 10,407
Operating expenses:				
Research and development	6,664	10,811	15,669	21,951
General and administrative	2,197	1,553	3,973	3,303
Gain on sale of kinase profiling services business		(565)		(1,120)
Total operating expenses	8,861	11,799	19,642	24,134
Income (loss) from operations	2,686	(6,625)	(1,503)	(13,727)
Other income (expense):				
Interest expense	(108)	(404)	(270)	(760)
Other income	5	1	12	1
Change in fair value of warrant and derivative liabilities	2,577	(269)	(1,380)	(816)
Total other income (expense), net	2,474	(672)	(1,638)	(1,575)
Income (loss) before income taxes	5,160	(7,297)	(3,141)	(15,302)
Provision for income tax			1	1
Consolidated net income (loss)	5,160	(7,297)	(3,142)	(15,303)
Net (income) loss attributable to redeemable non-controlling interest	(12)	150	61	248
Net income (loss) attributable to Ambit Biosciences Corporation	5,148	(7,147)	(3,081)	(15,055)
Other comprehensive income (loss):				
Foreign currency translation	3	(32)	(130)	1
Comprehensive income (loss)	$ 5,163	$ (7,329)	$ (3,272)	$ (15,302)
Net income (loss) per share attributable to common stockholders, basic	$ 0.45	$ (5,702.19)	$ (1.23)	$ (11,954.01)
Weighted average shares outstanding, basic	8,055,392	1,370	4,051,932	1,370
Net income (loss) per share attributable to common stockholders, diluted	$	$ (5,702.19)	$ (1.23)	$ (11,954.01)
Weighted average shares outstanding, diluted	9,751,585	1,370	4,051,932	1,370

See accompanying notes.

4

Table of Contents

Ambit Biosciences Corporation

Condensed Consolidated Statements of Cash Flows

(in thousands)

	Six Months Ended June 30,	
	2013	2012
	(unaudited)	
Operating activities		
Consolidated net loss	$ (3,142)	$ (15,303)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	219	635
Change in fair value of redeemable convertible preferred stock warrant and derivative liabilities	1,380	816
Noncash interest expense	92	278
Stock-based compensation	819	353
Gain on disposal of property and equipment	(11)	(4)
Deferred revenue	(13,724)	(3,172)
Gain on sale of kinase profiling services business		(1,120)
Changes in operating assets and liabilities:		
Accounts receivable		2,298
Prepaid expenses and other current assets	296	(72)
Accounts payable and accrued expenses	(1,335)	1,228
Accrued payroll and related expenses	10	(938)
Net cash used in operating activities	(15,396)	(15,001)
Investing activities		
Proceeds from sale of property and equipment	18	7
Purchase of property and equipment	(465)	(8)
Restricted cash	(63)	
Net cash used in investing activities	(510)	(1)
Financing activities		
Proceeds from issuance of common stock, net of offering costs	83,709	
Proceeds from issuance of put shares	2,725	
Proceeds from notes payable		8,000
Payments on notes payable	(2,593)	(2,365)
Net cash provided by financing activities	83,841	5,635
Effect of exchange rate changes on cash	(131)	
Net change in cash and cash equivalents	67,804	(9,367)
Cash and cash equivalents at beginning of the period	17,481	16,417
Cash and cash equivalents at end of the period	$ 85,285	$ 7,050
Supplemental disclosures of noncash investing and financing information		
Contributed capital related to cancelled Series D-2 financing warrants	$	$ 2,779
Conversion of redeemable non-controlling interest to common stock	$ 4,241	$

Conversion of preferred warrant liability to equity	$ 4,689	$
Conversion of preferred stock to common stock	$ 174,409	$
Supplemental disclosures of cash flow information		
Interest paid	$ 205	$ 506
Taxes paid	$ 1	$ 1

See accompanying notes.

Table of Contents

Ambit Biosciences Corporation

Notes to Condensed Consolidated Financial Statements

(Unaudited)

1. Organization and Summary of Significant Accounting Policies

Organization and Business

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

Initial Public Offering and Concurrent Private Placement

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

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

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

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

Principles of Consolidation

The consolidated financial statements include the accounts of the Company, its wholly-owned subsidiary Ambit Europe Limited (Ambit Europe) and its controlled subsidiary, Ambit Biosciences (Canada) Corporation (Ambit Canada), which became a wholly-owned subsidiary upon the Company s IPO. All intercompany transactions and balances are eliminated in consolidation. Ambit Europe was incorporated in England in June 2008. As of June 30, 2013, there have been no significant transactions related to Ambit Europe. Ambit Canada was formed in Canada in December 2004.

Consolidation of Ambit Canada s results included the following:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
	(in thousands)			
Research and development expense	$ (7)	$ (255)	$ (126)	$ (428)
Interest income (expense)	36	(10)	42	(10)

Net income (loss) of Ambit Canada	$ 29	$ (265)	$ (84)	$ (438)

Table of Contents

Income (loss) of Ambit Canada was allocated to the redeemable non-controlling interest based on the relative ownership of Ambit Canada. As of December 31, 2012, the redeemable non-controlling interest held 60% of the outstanding shares of Ambit Canada.

Reverse Stock Splits

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

Unaudited Interim Financial Information

The accompanying interim condensed consolidated financial statements are unaudited. These unaudited interim financial statements have been prepared in accordance with United States generally accepted accounting principles (GAAP) and following the requirements of the United States Securities and Exchange Commission (SEC) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP can be condensed or omitted. In management s opinion, the unaudited interim financial statements have been prepared on the same basis as the audited financial statements and include all adjustments, which include only normal recurring adjustments, necessary for the fair presentation of the Company s financial position and its results of operations and comprehensive loss and its cash flows for periods presented. These statements do not include all disclosures required by GAAP and should be read in conjunction with the Company s financial statements and accompanying notes for the fiscal year ended December 31, 2012, which is contained in the Company s final prospectus filed by the Company with the SEC on May 16, 2013 relating to the Company s Registration Statement on Form S-1/A (File No. 333-186760) for the Company s initial public offering. The results for interim periods are not necessarily indicative of the results expected for the full fiscal year or any other interim period.

Foreign Currency Translation and Transactions

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

Use of Estimates

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

Cash and Cash Equivalents

Cash and cash equivalents consist of cash and highly liquid investments, which include money market funds that are readily convertible into cash. The Company considers securities with remaining maturities of three months or less, at the date of purchase, to be cash equivalents. Cash and cash equivalents are recorded at face value or cost, which approximates fair market value.

Table of Contents

Fair Value of Financial Instruments

The carrying amounts of accounts payable and accrued liabilities are considered to be representative of their respective fair values because of the short-term nature of those instruments. Based on the borrowing rates currently available to the Company for loans with similar terms, the Company believes that the fair value of notes payable approximates its carrying value. The carrying amount of the warrant liabilities and redeemable non-controlling interest represent their fair values.

Warrant Liabilities

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

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

Revenues

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

Collaboration agreements entered into prior to 2011

For multiple-element agreements entered into prior to January 1, 2011 and not materially modified thereafter, such as the Company s agreement with Astellas Pharma Inc., the Company analyzed the agreement to determine whether the elements within the agreement could be separated or whether they must be accounted for as a single unit of accounting. If the delivered element, which for the Company is commonly a license, had stand-alone value and the fair value of the undelivered elements, which for the Company was generally collaborative research activities, could be determined, the Company recognized revenue separately under the residual method as the elements under the agreement were delivered. If the delivered element did not have stand-alone value or if the fair value of the undelivered element could not be determined, the agreement was then accounted for as a single unit of accounting, with consideration received under the agreement recognized as revenue on the straight-line basis over the estimated period of performance, which for the Company was generally the expected term of the research and development plan.

Milestones

Revenue from milestones is recognized when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved, provided that the milestone event is substantive. A milestone event is considered to be substantive if its achievability was not reasonably assured at the

Table of Contents

inception of the arrangement and the Company s efforts led to the achievement of the milestone (or if the milestone was due upon the occurrence of a specific outcome resulting from the Company s performance). Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty s performance are not considered to be milestone events. If both of these criteria are not met, the milestone payment is recognized over the remaining minimum period of the Company s performance obligations under the arrangement. The Company assesses whether a milestone is substantive at the inception of each arrangement.

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

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

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

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

The Company has recognized the following revenue from collaboration agreements:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
	(in thousands)			
Upfront licensing fees	$ 9,872	$ 1,586	$ 13,724	$ 3,172
Collaborative research activities	1,675	3,588	4,415	7,235
Total revenue from collaboration agreements	$ 11,547	$ 5,174	$ 18,139	$ 10,407

Deferred revenue

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

Stock-Based Compensation

Stock-based compensation expense represents the cost of the grant date fair value of employee stock option grants recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis, net of estimated forfeitures. For stock option grants with performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable or the performance condition has been achieved. The Company determines equity-based compensation using the Black-Scholes option pricing model.

9

Table of Contents

Total stock-based compensation was allocated as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
	(in thousands)			
Research and development	$ 124	$ 29	$ 235	$ 68
General and administrative	297	117	584	285
	$ 421	$ 146	$ 819	$ 353

As of June 30, 2013, total unrecognized stock-based compensation costs related to non-vested stock options was approximately $3.5 million and the weighted-average period over which it is expected to be recognized is approximately 3.3 years.

Net Income (Loss) Per Share Attributable to Common Stockholders

Prior to the IPO, the Company had common stock and participating preferred stock outstanding. The Company applies the two-class method for calculating net income (loss) per share since it has issued securities, other than common stock, that contractually entitle the holder to participate in dividends and earnings of the Company.

Basic earnings per common share is calculated by dividing net earnings (loss) available to common stock holders by the weighted average number of shares outstanding, without consideration for common stock equivalents. All undistributed earnings are allocated first to the preferred stockholders based on their contractual right to dividends. This right is calculated on a pro rated basis for the portion of the period the preferred shares were outstanding. Any remaining undistributed earnings are allocated between preferred and common stock on a weighted average basis.

For the Company s diluted earnings per common share calculation, the Company uses the two-class method. This calculation does not assume that preferred shares are converted into common shares. Both the numerator and the denominator are adjusted for the impact of the redeemable non-controlling interest, warrants to purchase preferred shares and warrants to purchase common shares because their effect was dilutive. The undistributed earnings after the adjustment for the effect of the dilutive securities is allocated first to the preferred stockholders based on their contractual right to dividends. This right was calculated on a pro rated basis for the portion of the period the preferred shares were outstanding. Any remaining undistributed earnings are allocated between preferred and common stock on a weighted average basis. In all periods presented, the Company s outstanding stock options were excluded from the calculation of earnings (loss) per share because the effect would be antidilutive.

For periods in which the Company has a net loss, such as for the three months ended June 30, 2012 and the six months ended June 30, 2013 and 2012, basic and diluted earnings per share are the same. Consequently, diluted earnings per share for these periods are not presented separately in the table below.

Table of Contents

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Numerator for basic income (loss) per share:				
Income (loss) attributable to Ambit Biosciences Corporation	$ 5,148	$ (7,147)	$ (3,081)	$ (15,055)
Accretion to redemption value of redeemable convertible preferred stock	(1,315)	(441)	(3,634)	(881)
Change in fair value of redeemable non-controlling interest	3,246	(224)	1,747	(441)
Net income allocated to participating preferred stockholders	(3,457)			
Net income (loss) available to common stockholders	$ 3,622	$ (7,812)	$ (4,968)	$ (16,377)
Numerator for diluted income per share:				
Income attributable to Ambit Biosciences Corporation	$ 5,148			
Accretion to redemption value of redeemable convertible preferred stock	(1,315)			
Net income allocated to redeemable non-controlling interest	12			
Change in fair value of warrants for purchase of convertible preferred stock	(743)			
Change in fair value of warrants for purchase of common stock	(1,748)			
Net income allocated to participating preferred stockholders	(1,354)			
Net income available to common stockholders	$			
Denominator for basic and diluted income (loss) per share:				
Weighted average shares for basic EPS	8,055,392	1,370	4,051,932	1,370
Weighted average effect of dilutive securities	1,696,193			
Weighted average shares for diluted EPS	9,751,585			
Basic EPS	$ 0.45	$ (5,702.19)	$ (1.23)	$ (11,954.01)
Diluted EPS	$	$ (5,702.19)	$ (1.23)	$ (11,954.01)

Table of Contents

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Convertible preferred stock outstanding		4,014,981		4,014,981
Redeemable non-controlling interest		273,264		273,264
Warrants for convertible preferred stock		654,829		654,829
Warrants for common stock		1,057	1,635,283	1,057
Convertible notes payable		481,777		481,777
Common stock options	1,264,086	5,925	1,264,086	5,925
	1,264,086	5,431,833	2,899,369	5,431,833

2. Ambit Canada

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

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

The redeemable non-controlling interest was initially valued using the fair value of the Series C-2, Series D, Series D-2 and Series E redeemable convertible preferred stock. At each reporting period, the Company adjusted the carrying value of the redeemable non-controlling interest by the net income (loss) attributable to the redeemable non-controlling interest. Any difference between the fair value and the adjusted carrying value of the redeemable non-controlling interest was recorded as an adjustment to additional paid-in capital and presented as a component of net loss attributable to common stockholders in the accompanying condensed consolidated statements of comprehensive income (loss). The redeemable non-controlling interest was measured at fair value until the IPO, at which time no Class C-2, Series D, Series D-2 or Class E shares of Ambit Canada were held by GrowthWorks or any other third party. The redeemable non-controlling interest was reclassified to additional paid-in capital.

Income (loss) of Ambit Canada was allocated to the redeemable non-controlling interest based on the relative ownership of Ambit Canada. As of December 31, 2012 and June 30, 2013, the redeemable non-controlling interest held 60%, and 0%, respectively, of the outstanding shares of Ambit Canada.

Table of Contents

3. Fair Value Measurements

The following tables and disclosure present information about the Company s financial assets and financial liabilities measured at fair value on a recurring basis as of June 30, 2013 and December 31, 2012, and indicates the fair value hierarchy of the valuation techniques utilized by the Company to determine such fair value. As a basis for categorizing inputs, the Company uses a three-tier fair value hierarchy, which prioritizes the inputs used to measure fair value from market based assumptions to entity specific assumptions:

Level 1: Observable inputs such as quoted prices in active markets;

Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and

Level 3: Unobservable inputs in which there is little or no market data, which requires the entity to develop its own assumptions.

As of June 30, 2013 and December 31, 2012, cash and cash equivalents are measured at face value which approximates fair value and are classified within the Level 1 designation discussed above. Financial assets and liabilities that are measured or disclosed at fair value on a recurring basis, and are classified within the Level 3 designation include the preferred stock and common stock warrant liabilities and the redeemable non-controlling interest. None of the Company s non-financial assets and liabilities are recorded at fair value on a non-recurring basis.

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

The following weighted-average assumptions were used in determining the fair value of the outstanding preferred stock and common stock warrant liabilities valued using the Black-Scholes option pricing model as of June 30, 2013 and December 31, 2012:

	June 30, 2013	December 31, 2012
Risk-free interest rate	2.3%	1.6%
Expected dividend yield	0.0%	0.0%
Expected volatility	63.0%	63.1%
Expected term in years	9.3	9.2

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

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

Table of Contents

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

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

	Common Warrant Liabilities	Preferred Warrant Liabilities	Redeemable Non-Controlling Interest
		(in thousands)	
Balance at December 31, 2012	$ 6,182	$ 4,358	$ 3,323
Issuance of shares of redeemable non-controlling interest			2,725
Change in fair value	1,049	331	(1,747)
Net loss attributable to redeemable non-controlling interest			(61)
Exercise of common warrant liabilities	(266)		
Reclassification to additional paid-in capital upon the closing of the Company s IPO		(4,689)	(4,240)
Balance at June 30, 2013	$ 6,965	$	$

4. Warrants

In May 2013, in connection with the IPO, all outstanding preferred stock warrants converted into warrants to purchase shares of common stock. The Company s outstanding warrant liabilities consisted of the following:

		June 30, 2013		
Issue Date	Expiration Date	Exercise Price per Share	Shares Issuable upon Exercise	Fair Value
		(in thousands, except share and per share data)		
October 2012	October 2022	$ 0.24	1,017,227	$ 6,943
November 2012	October 2022	0.24	3,324	22
			1,020,551	$ 6,965

Table of Contents

The following table summarizes the warrants outstanding for purchase of common stock as of June 30, 2013 (excluding the warrants above that require liability accounting):

Shares Issuable upon Exercise	Exercise Price	Expiration Date
366	$ 103.20	October 2013
300	103.20	December 2013
454	103.20	July 2014
96	103.20	October 2014
70	103.20	December 2014
126	103.20	March 2015
100	103.20	June 2015
9,688	103.20	October 2015
218	54.99	August 2016
3,875	103.20	September 2017
70,704	21.84	June 2019
2,266	21.84	July 2019
78	2,184.00	July 2019
85,714	16.80	March 2020
20,690	36.96	September 2020
39	3,696.00	September 2020
419,948	0.02	May 2021
614,732		

5. Notes Payable

Venture Loans

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

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

As of June 30, 2013 and December 31, 2012 the outstanding principal balance of the Venture Loans was $1.8 million and $4.4 million, respectively, which was offset by outstanding debt discount of $21,000 and $92,000, respectively.

Table of Contents

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

Upon the closing of the IPO in May 2013, 133,807,619 outstanding shares of redeemable convertible preferred stock and 1,590,014 shares of convertible preferred stock were converted into 6,449,073 shares of common stock, and the related carrying values of $160.7 million and $13.7 million were reclassified to additional paid-in capital. At June 30, 2013, no shares of convertible preferred stock were issued or outstanding.

7. Collaboration Agreements

Astellas Pharma Inc. and Astellas US LLC

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

On March 7, 2013 Astellas exercised the right to terminate the Company s agreement, effective September 3, 2013. Until September 3, 2013 Astellas and the Company will continue to share agreed-upon development costs equally. Subsequent to September 3, 2013, the Company will be solely responsible for development costs associated with quizartinib.

Table of Contents

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

The following discussion and analysis should be read in conjunction with our financial statements and accompanying notes included in this Quarterly Report on Form 10-Q and the financial statements and accompanying notes thereto for the fiscal year ended December 31, 2012 and the related Management s Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our final prospectus filed by us with the Securities and Exchange Commission, or SEC, on May 16, 2013 relating to our Registration Statement on Form S-1/A (File No. 333-186760) for our initial public offering.

Forward-Looking Statements

This discussion contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward looking statements, which represent our intent, belief, or current expectations, involve risks and uncertainties. We use words such as may, will, expect, anticipate, estimate, intend, plan, predict, potential, believe, should and similar expressions to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Although we believe the expectations reflected in these forward-looking statements are reasonable, such statements are inherently subject to risk and we can give no assurances that our expectations will prove to be correct. You should not place undue reliance on these forward-looking statements, which apply only as of the date of this Quarterly Report on Form 10-Q. As a result of many factors, including without limitation those set forth under Risk Factors under Item 1A of Part II below, and elsewhere in this Quarterly Report on Form 10-Q, our actual results may differ materially from those anticipated in these forward-looking statements. We undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of drugs to treat unmet medical needs in oncology, autoimmune and inflammatory diseases by inhibiting kinases that are important drivers for those diseases. Our pipeline currently includes three programs, each aimed at the inhibition of validated kinase targets. Our lead drug candidate, quizartinib, which we formerly referred to as AC220, is a once-daily, orally-administered, potent and selective inhibitor of the FMS-like tyrosine kinase 3, or FLT3. Quizartinib is currently in Phase 2b clinical development in patients with relapsed/refractory acute myeloid leukemia, or AML, who express a genetic mutation in FLT3. Our second drug candidate in clinical development, AC410, is a potent, selective, orally-administered, small molecule inhibitor of Janus kinase 2, or JAK2, that has potential utility for the treatment of autoimmune and inflammatory diseases. Our third program consists of a potent and exquisitely selective small molecule compound, AC708, which inhibits the colony-stimulating factor-1 receptor, or CSF1R, a receptor tyrosine kinase. This compound is in preclinical studies and has potential utility in oncology, autoimmune and inflammatory diseases. All of our drug candidates and clinical candidates have been internally discovered by us.

We have no products approved for sale, we have not generated any revenues from product sales and we have incurred significant operating losses since our inception. We have generated revenues from upfront payments and reimbursements associated with our collaboration agreements and from our former kinase profiling services business. We have never been profitable and have incurred consolidated net losses of approximately $3.1 million for the six months ended June 30, 2013. As of June 30, 2013, we had an accumulated deficit of $240.1 million. We expect to continue to incur significant operating losses and negative cash flows from operating activities for the foreseeable future as we continue the clinical development of quizartinib, seek regulatory approval for and, if approved, pursue eventual commercialization of quizartinib, and advance our other drug candidates through preclinical studies and clinical trials.

We conduct our activities through Ambit Biosciences Corporation, a Delaware corporation, from our primary facility in San Diego, California. Additionally, as of June 30, 2013, we have a wholly-owned subsidiary, Ambit Canada, which in the past conducted limited research and development activities in Toronto. We also have a wholly-owned subsidiary, Ambit Europe Limited, located in the United Kingdom, which has limited operations related to regulatory filings in the European Union. The following information is presented on a consolidated basis to include the accounts of these subsidiaries. All intercompany transactions and balances are eliminated in consolidation.

Table of Contents

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

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

Financial Overview

Revenues

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

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

Research and Development Expenses

The majority of our operating expenses to date have been incurred in research and development activities. Research and development expenses relate primarily to the discovery and development of our drug candidates. Our business model is dependent upon our continuing to conduct a significant amount of research and development. To date, quizartinib represents the largest portion of our research and development expense. From the date of our agreement with Astellas and through the effective date of the termination, we share equally in any agreed-upon research and development costs for quizartinib and any follow-on compounds in the United States and European Union and Astellas is solely responsible for development costs outside of the United States and European Union. Following the effective date of the termination, we will be responsible for all world-wide development costs for quizartinib and any follow-on compounds. Our research and development expenses consist primarily of:

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

stock-based compensation expense to employees and consultants; and

costs associated with other research activities and regulatory approvals.

Research and development costs are expensed as incurred.

Table of Contents

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

	Three Months Ended June 30,		Six Months Ended June 30,		Total January 1, 2007 through June 30,
	2013	2012	2013	2012	2013
Quizartinib	$ 4,895	$ 8,178	$ 11,887	$ 16,438	$ 117,313
AC410 /AC430	12	257	50	714	15,673
CSF1R	522	333	873	802	12,202
Discovery projects	556	1,129	1,228	2,248	59,771
R&D administration	679	914	1,631	1,749	15,043
Total	$ 6,664	$ 10,811	$ 15,669	$ 21,951	$ 220,002

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

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

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

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

- per patient trial costs;
- the number of patients that participate in the trials;
- the number of sites included in the trials;
- the countries in which the trial is conducted;
- the length of time required to enroll eligible patients;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;

potential additional safety monitoring or other studies requested by regulatory agencies;

the duration of patient follow-up; and

the efficacy and safety profile of the drug candidate.

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

Table of Contents

General and Administrative Expenses

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

We anticipate that our general and administrative expenses will continue to be significant and will increase as a result of being a public company and associated increased payroll, expanded infrastructure and higher consulting, legal, accounting and investor relations costs, and director and officer insurance premiums. We expect these increases to be partially offset by a reduction in costs related to a facility move completed in the first quarter of 2013.

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

Interest Expense

Interest expense consists primarily of coupon interest, amortization of debt discount and amortization of deferred financing costs associated with our 2012 bridge loan, our equipment notes payable and our venture loans, as described in Notes 6 and 7 to our audited consolidated financial statements in our Registration Statement on Form S-1 (File No. 333-186760).

Other Income

Other income consists primarily of: (i) interest income earned on our cash and cash equivalents; and (ii) exchange rate gains and losses on transactions denominated in a currency other than our functional currency, the U.S. dollar.

Critical Accounting Policies and Significant Judgments and Estimates

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

We discussed accounting policies and assumptions that involve a higher degree of judgment and complexity within Note 1 to our audited consolidated financial statements in our Registration Statement on Form S-1 (File No. 333-186760). There have been no material changes to our critical accounting policies and estimates as disclosed in our Registration Statement on Form S-1 (File No. 333-186760).

Results of Operations

Comparison of the Three Months Ended June 30, 2013 and 2012

Collaboration Agreement Revenues. We recorded revenues of $11.5 million and $5.2 million for the three months ended June 30, 2013 and 2012, respectively, under our agreement with Astellas. The increase of approximately $6.4 million was primarily due to an increase in license fee amortization revenue being recognized during the three months ended June 30, 2013. In March 2013, we received a notice of termination of the agreement from Astellas, which termination will be effective in September 2013. Accordingly, the remaining deferred revenue related to the upfront license fee is being recognized over the period from March 2013 through September 2013. This increase was partially offset by a reduction in cost reimbursement revenue due to lower quizartinib research and development expenses. The reduction in quizartinib research and development expenses was due to a reduction in the number of patients being treated and followed in our Phase 2 clinical trial.

20

Table of Contents

Research and Development Expenses. Our research and development expenses were $6.7 million and $10.8 million for the three months ended June 30, 2013 and 2012, respectively. A comparison of research and development expenses by category is as follows (in thousands):

	Three Months Ended June 30,		Increase/ (Decrease)
	2013	2012	
Outside services	$ 4,377	$ 8,197	$ (3,820)
Salaries and personnel	1,805	1,660	145
Facilities and operations	482	954	(472)
Total	$ 6,664	$ 10,811	$ (4,147)

Outside Services. Expenses for outside services, such as for CROs and investigator sites, decreased approximately $3.8 million from $8.2 million for the three months ended June 30, 2012 to $4.4 million for the three months ended June 30, 2013. This decrease was due to lower quizartinib research and development expenses, resulting from a reduction in the number of patients being treated and followed in our Phase 2 clinical trial.

Facilities and Operations. Expenses for facilities and operations decreased approximately $472,000 from $954,000 for the three months ended June 30, 2012 to $482,000 for the three months ended June 30, 2013. The decrease was primarily due to a decrease in our rent expense, as our monthly rent expense for our Sorrento Valley Boulevard facility was reduced to approximately $55,000 per month effective July 2012.

General and Administrative Expenses. Our general and administrative expenses were $2.2 million and $1.6 million for the three months ended June 30, 2013 and 2012, respectively. The increase of approximately $644,000 was due to increased costs related to patent prosecution and increased stock-based compensation expense.

Interest Expense. Interest expense decreased approximately $296,000 from $404,000 for the three months ended June 30, 2012 to $108,000 for the three months ended June 30, 2013. The decrease in interest expense was due to the decrease in the principal balance of our outstanding venture loan as we pay down more principal near the maturity of the loan in October 2013.

Change in Fair Value of Warrant Liabilities. During the three months ended June 30, 2013 and 2012, the change in fair value of the stock warrant liabilities related primarily to changes in the fair value of the underlying preferred or common securities. The change in fair value for the three months ended June 30, 2013 was $2.6 million due primarily to the impact of common stock value decreases on the 1.1 million common stock warrants issued in October 2012 in connection with our Series E preferred stock financing.

Comparison of the Six Months Ended June 30, 2013 and 2012

Collaboration Agreement Revenues. We recorded revenues of $18.1 million and $10.4 million for the six months ended June 30, 2013 and 2012, respectively, under our agreement with Astellas. The increase of approximately $7.7 million was primarily due to an increase in license fee amortization revenue being recognized during the six months ended June 30, 2013. In March 2013, we received a notice of termination of the agreement from Astellas, which termination will be effective in September 2013. Accordingly, the remaining deferred revenue related to the upfront license fee is being recognized over the period from March 2013 through September 2013. This increase was partially offset by a reduction in cost reimbursement revenue due to lower quizartinib research and development expenses. The reduction in quizartinib research and development expenses was due to a reduction in the number of patients being treated and followed in our Phase 2 clinical trial.

Table of Contents

Research and Development Expenses. Our research and development expenses were $15.7 million and $22.0 million for the six months ended June 30, 2013 and 2012, respectively. A comparison of research and development expenses by category is as follows (in thousands):

	Six Months Ended June 30,		
	2013	2012	Decrease
Outside services	$ 11,085	$ 16,810	$ (5,725)
Salaries and personnel	3,509	3,320	189
Facilities and operations	1,075	1,821	(746)
Total	$ 15,669	$ 21,951	$ (6,282)

Outside Services. Expenses for outside services, such as for CROs and investigator sites, decreased approximately $5.7 million from $16.8 million for the six months ended June 30, 2013 to $11.1 million for the six months ended June 30, 2012. The decrease was due to lower quizartinib research and development expenses, resulting from a reduction in the number of patients being treated and followed in the Phase 2 clinical trial.

Facilities and Operations. Expenses for facilities and operations decreased approximately $746,000 from $1.8 million for the six months ended June 30, 2012 to $1.1 million for the six months ended June 30, 2013. The decrease was primarily due to a decrease in our rent expense, as our monthly rent expense for our Sorrento Valley Boulevard facility was reduced to approximately $55,000 per month effective July 2012.

General and Administrative Expense. General and administrative expenses increased approximately $670,000 from $3.3 million for the six months ended June 30, 2012 to $4.0 million for the six months ended June 30, 2013. The increase was primarily due to was due to increased costs related to patent prosecution and increased stock-based compensation expense.

Interest Expense. Interest expense decreased approximately $490,000 from $760,000 for the six months ended June 30, 2012 to $270,000 for the six months ended June 30, 2013. The decrease in interest expense was primarily due to the decrease in the principal balance of our outstanding venture loan as we pay down more principal near the maturity of the loan.

Change in Fair Value of Warrant and Derivative Liabilities. During the six months ended June 30, 2013 and 2012, the change in fair value of the stock warrant liabilities related primarily to increases in the fair value of the underlying preferred or common securities. The change in fair value for the six months ended June 30, 2013 was $564,000 higher than the change for the six months ended June 30, 2012 due primarily to the impact of common stock value increase on the 1.1 million common stock warrants issued in October 2012 in connection with our Series E preferred stock financing.

Liquidity and Capital Resources

We have incurred losses substantially since inception and negative cash flows from operating activities for the six months ended June 30, 2013. As of June 30, 2013, we had an accumulated deficit of $240.1 million. We anticipate that we will continue to incur net losses for the foreseeable future as we: (i) continue the development and potential commercialization of our lead drug candidate, quizartinib, (ii) continue our research and development programs to advance our internal product pipeline and (iii) incur additional costs associated with being a public company.

From our inception through June 30, 2013, we have funded our consolidated operations primarily through the placements of equity and convertible debt securities and upfront payments from our collaboration agreements. Additionally, we have funded a portion of our operations from service revenues and additional funding under our collaboration agreements. As of June 30, 2013, we had cash and cash equivalents of approximately $85.3 million.

Table of Contents

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

	Six Months Ended June 30,	
	2013	2012
Net cash used in operating activities	$ (15,396)	$ (15,001)
Net cash used in investing activities	(510)	(1)
Net cash provided by financing activities	83,841	5,635
Effect of exchange rate changes on cash	(131)	
Net increase (decrease) in cash and cash equivalents	$ 67,804	$ (9,367)

Cash used in operating activities increased $395,000 from $15.0 million for the six months ended June 30, 2012 to $15.4 million for the six months ended June 30, 2013. Our net loss decreased approximately $12.2 million from $15.3 million for the six months ended June 30, 2012 to $3.1 million for the six months ended June 30, 2013. This decrease was offset by an increase in the usage of deferred revenue of approximately $10.6 million from $3.2 million for the six months ended June 30, 2012 to $13.7 million for the six months ended June 30, 2013. Non-cash expenses increased approximately $1.5 million from $958,000 for the six months ended June 30, 2012 to $2.5 million for the six months ended June 30, 2013. Changes in working capital and deferrals excluding deferred revenue in the six months ended June 30, 2013 and 2012 used cash of $1.0 million and provided cash of $2.5 million, respectively.

During the six months ended June 30, 2013, investing activities used cash of $510,000, primarily due to purchases of property and equipment. During the six months ended June 30, 2012, investing activities used cash of $1,000, primarily due to purchases of property and equipment.

Financing activities provided cash of $83.8 million and $5.6 million for the six months ended June 30, 2013 and 2012, respectively. Cash provided during the six months ended June 30, 2013 was primarily derived from our IPO and concurrent private offering. Cash provided during the six months ended June 30, 2012 was primarily derived from the issuance of a note payable.

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

Operating Capital Requirements

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

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

Table of Contents

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

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

Our commitment for long-term debt relates primarily to the $12.0 million venture loan executed in March 2010, of which $4.3 million was outstanding as of December 31, 2012 and $1.8 million was outstanding as of June 30, 2013.

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

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

- the progress, costs and results of our Phase 2b clinical trial, our anticipated Phase 3 clinical trial and future trials that may be required to support regulatory approval and label expansion for quizartinib;
- the outcome, timing and cost of regulatory approvals;
- the initiation, progress, timing and results of preclinical studies and clinical trials for any of our other drug candidates;
- the costs and timing of establishing sales, marketing and distribution capabilities;
- delays that may be caused by changing regulatory requirements;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims; and
- the extent to which we acquire or invest in businesses, products or technologies.

We believe our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations through at least the next 12 months and will be sufficient to fund the continued development of quizartinib through receipt of topline data from our planned Phase 3 clinical trial in patients with relapsed/refractory AML. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially.

Off-Balance Sheet Arrangements

As of June 30, 2013 and 2012, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as special purpose or structured finance entities, which would have been established for the purposes of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. As such, we are not materially exposed to any financing, liquidity, market or credit risk that could arise if we had engaged in these relationships.

24

Table of Contents

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

Our cash and cash equivalents as of June 30, 2013 consisted of cash and money market funds. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition and/or results of operation.

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

Foreign Currency Risk

Our balance sheet as of June 30, 2013 includes cash and cash equivalent balances of $16,000 denominated in Canadian dollars through our Canadian subsidiary, Ambit Canada. The majority of Ambit Canada s operational activities are denominated in Canadian dollars. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate losses during the three- and six-month periods ended June 30, 2013 and 2012.

Item 4. Controls and Procedures

Disclosure Controls and Procedures

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

As of June 30, 2013, we carried out an evaluation, under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Based on this evaluation, our chief executive officer and chief financial officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of June 30, 2013.

An evaluation was also performed under the supervision and with the participation of our management, including our chief executive officer and our chief financial officer, of any change in our internal control over financial reporting that occurred during our last fiscal quarter and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. That evaluation did not identify any change in our internal control over financial reporting that occurred during our latest fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors

You should carefully consider the following risk factors, as well as the other information in this report, before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business. We have marked with an asterisk (*) those risk factors that reflect changes from the risk factors included in our final prospectus filed with the Securities and Exchange Commission on May 16, 2013 relating to our Registration Statement on Form S-1 (File No. 333-186760) for our initial public offering.

Risks Related to Our Business and Industry

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

Our future success is substantially dependent on our ability to obtain regulatory approval for, and then successfully commercialize quizartinib, our lead drug candidate, for which a Phase 2b clinical trial is ongoing. Our other drug candidates are in earlier stages of development. Our business depends entirely on the successful development and commercialization of our drug candidates. We have not completed the development of any drug candidates, we currently generate no revenues from sales of any drugs, and we may never be able to develop a marketable drug.

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

We expect, pending regulatory authority input, to initiate a randomized, comparative Phase 3 clinical trial of quizartinib in patients with relapsed/refractory acute myeloid leukemia, or AML, in early 2014. There is no guarantee that this trial will commence or be completed on time or at all. Even if the trial is successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do. To the extent that the results of the trial are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant additional resources to conduct additional trials in support of potential approval of quizartinib.

We have initiated discussions with the FDA regarding the acceptability of the primary endpoint in our Phase 2 and Phase 2b clinical trials (CRc) as a novel surrogate endpoint that could support an accelerated approval of quizartinib based upon our Phase 2 and Phase 2b clinical trials. There is no guarantee that the FDA will accept CRc as a surrogate endpoint or, even if accepted, that our Phase 2 and Phase 2b data would be sufficient to support regulatory approval on an accelerated basis.

We cannot anticipate when or if we will seek regulatory review of quizartinib for any other indications. We have not previously submitted a New Drug Application, or NDA, to the FDA, or similar drug approval filings to comparable foreign authorities, or received marketing approval for any drug candidate, and we cannot be certain that quizartinib will be successful in clinical trials or receive regulatory approval for any indication. If we do not receive regulatory approvals for and successfully commercialize quizartinib on a timely basis or at all, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market quizartinib, our revenues

Table of Contents

will be dependent, in part, on our collaborator s ability to obtain regulatory approval of the companion diagnostic to be used with quizartinib, our collaborator s ability to commercialize the test as well as the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for the treatment of AML are not as significant as we estimate, our business and prospects will be harmed.

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

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

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

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

Our collaboration with Astellas for the development of quizartinib terminates in September 2013. Any interruptions or delays in transitioning full responsibility for clinical development to us in connection with such termination could delay the commencement of the Phase 3 clinical trial. In addition, any delays in obtaining data from the drug-drug interaction study or in the analysis of data from the Phase 2b clinical trial could delay the commencement of the Phase 3 clinical trial. We are currently evaluating quizartinib in two other indications in AML and plan, in the future, to initiate additional clinical trials in AML and other indications. We do not know whether ongoing clinical trials will be completed on schedule or at all, or whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

- obtaining regulatory approval to commence a trial;
- reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining institutional review board approval at each clinical trial site;
- recruiting suitable patients to participate in a trial;
- developing and validating the companion diagnostic to be used in the trial on a timely basis;
- having patients complete a trial or return for post-treatment follow-up;

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

adding new clinical trial sites; or

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

Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians and patients perceptions as to the potential

Table of Contents

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

We could encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our drug candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, our collaborators, the institutional review boards, or IRBs, in the institutions in which such trials are being conducted, the Data Safety Monitoring Board, or DSMB, for such trial, or by the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our drug candidates, the commercial prospects of our drug candidates will be harmed, and our ability to generate product revenues from any of these drug candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly. Furthermore, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our drug candidates.

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

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

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

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a drug candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a drug candidate s clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our drug candidates may not be sufficient to the satisfaction of FDA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or foreign jurisdictions, on an accelerated basis or otherwise;

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

Table of Contents

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

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

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

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

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

In March 2013, Astellas exercised the right to terminate our collaboration agreement, effective in September 2013, under which we collaborated with Astellas for the development of quizartinib and under which we and Astellas shared agreed-upon development costs equally. As a result of the termination of our collaboration, we will be solely responsible for developing and commercializing quizartinib within the United States and the rest of the world and will be responsible for many of the functions previously expected to be Astellas responsibility, including management and oversight of certain ongoing clinical trials and the planned Phase 3 clinical trial, as well as submitting the NDA for quizartinib to the FDA. We and Astellas are in the process of transitioning the development activities currently being conducted by Astellas to us. We cannot assure you that our efforts to transition Astellas collaboration responsibilities will be completed on a timely basis, or at all. If we are unable to successfully complete the transition of Astellas quizartinib development activities to us on a timely basis, our development plans may be delayed, which could harm our business, prospects, financial condition and results of operations.

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

We intend to initially seek approval of quizartinib in relapsed/refractory AML patients with internal tandem duplication, or ITD, mutations in the FMS-like tyrosine kinase 3, or FLT3, gene, which we refer to as FLT3-ITD positive. The initial proposed drug label being sought for quizartinib specific to this patient population would indicate a potential for enhanced efficacy and/or a greater likelihood of a positive response in patients that carry the FLT3-ITD positive genotype. Accordingly, it is expected that the Phase 3 trial designed to support marketing approval for quizartinib will use a diagnostic test to select patients that are FLT3-ITD positive. In the United States, the FDA requires that the diagnostic test used to select patients in a pivotal trial be approved in parallel with the drug candidate as a companion diagnostic. A companion diagnostic is an in vitro diagnostic device that provides information that is essential for the safe and effective use of a corresponding therapeutic product. We believe a companion diagnostic to test for the FLT3-ITD positive genotype will be required for the approval of quizartinib. Companion diagnostics are subject to regulation as medical devices by the FDA and may be subject to regulation by comparable regulatory authorities in various foreign countries. The process of complying with the requirements of the FDA and comparable foreign agencies to support marketing authorization of a companion diagnostic is costly, time consuming and burdensome.

We do not develop companion diagnostics internally and thus we are dependent on the sustained cooperation and effort of third parties in developing and obtaining approval for these companion diagnostics. We have entered

Table of Contents

into an agreement with Genoptix, pursuant to which Genoptix will be responsible for developing the companion diagnostic and obtaining marketing authorization from the FDA. We believe Genoptix will need to submit a premarket approval application, or PMA, for such test, which we anticipate will happen in parallel with our submission of an NDA for quizartinib in accordance with FDA guidance that a novel therapeutic product and companion diagnostic device should generally be developed and approved contemporaneously to support the therapeutic product s safe and effective use. We currently do not believe that any clinical trials other than the quizartinib Phase 3 clinical trial will be required to support the PMA for the companion diagnostic. However, the FDA may require Genoptix to perform further tests requiring access to patient samples for the test submission and/or future products. We intend to provide access to patient samples to Genoptix for such purposes and our informed consents with patients allow us to permit a third party to test these samples, as required.

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

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

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

To date, the clinical development program for quizartinib includes over 400 patients treated in our Phase 1 and Phase 2 clinical trials in relapsed/refractory AML. The adverse events we have observed to date are manageable and the most common all grade treatment-emergent adverse events (reported in ³ 20% of subjects) in our Phase 2 clinical trials included gastrointestinal toxicities, fatigue, anemia, QT prolongation (changes in the patient s electrocardiogram pattern), and dysgeusia (distortion of the sense of taste). Overall, there were no major differences between safety findings in FLT3-ITD positive and FLT3-ITD negative patients or between the Phase 1 and Phase 2 clinical trials. QT prolongation is a common adverse event associated with multiple other kinase inhibitors and may be a class effect. The majority of cases of QT prolongation with quizartinib are asymptomatic and occur within the first month of treatment. Additionally, the majority of patients that experienced QT prolongation did not discontinue quizartinib treatment due to this adverse event. Nonetheless, QT prolongation may be associated with changes in electric conduction in the heart and may cause irregularities of the heart beat which could be potentially serious, life-threatening or fatal and require ECG monitoring and treatment. To date, there has been one case of Grade 4 QT interval prolongation with Torsade de pointes (an abnormal cardiac rhythm) in a patient taking quizartinib with multiple concomitant medications in our Phase 2 clinical trial. Results of our current and anticipated trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials

Table of Contents

could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our drug candidates for any or all targeted indications. In addition, the drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

Additionally if quizartinib or any of our other drug candidates receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh its risks, which in the case of quizartinib may include, among other things, a medication guide outlining the risks of QT prolongation for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by the product, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way quizartinib is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

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

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

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

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

The FDA has substantial discretion in the approval process and may either refuse to consider our application for substantive review or may form the opinion after review of our data that our application is insufficient to allow approval of our drug candidates. If the FDA does not consider or approve our application, it may require that we conduct additional clinical, preclinical or manufacturing validation studies and submit that data before it will reconsider our application. Depending on the extent of these or any other studies, approval of any applications that we submit may be delayed by several years, or may require us to expend more resources than we have available. It is also possible that additional studies, if

performed and completed, may not be successful or considered sufficient by the FDA to support approval. If any of these outcomes occur, we may be forced to abandon one or more of our applications for approval, which might significantly harm our business, prospects, financial condition and results of operations.

Table of Contents

Even if we do receive regulatory approval to market a drug candidate, any such approval may be subject to limitations on the indicated uses for which we may market the product. It is possible that none of our existing drug candidates or any drug candidates we may seek to develop in the future will ever obtain the appropriate regulatory approvals necessary for us or our collaborators to commence product sales. Any delay in obtaining, or an inability to obtain, applicable regulatory approvals would prevent us from commercializing our drug candidates, generating revenues and achieving and sustaining profitability.

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

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

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

Any regulatory approvals that we or our strategic partners receive for our drug candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the drug candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our drug candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices, or cGMPs, and current good clinical practices, or cGCPs, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

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

Table of Contents

fines, warning letters or holds on clinical trials;

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

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

injunctions or the imposition of civil or criminal penalties.

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

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

We have agreements with third-party CROs to conduct or monitor and manage data for our ongoing preclinical and clinical programs, including our ongoing Phase 2 clinical trials for quizartinib. We anticipate that we will engage one or more third party CROs in connection with our planned Phase 3 clinical trial. We rely heavily on these parties for execution of our preclinical and clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for all of our drug candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our or our respective CROs failure to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies or other drug development activities, which could harm our competitive position. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize our drug candidates. As a result, our financial results and the commercial prospects for quizartinib and our other drug candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

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

33

Table of Contents

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

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

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

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our products or in the manufacturing facilities in which our products are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability or other issues relating to the manufacture of any of our products will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide any drug candidates to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely. In addition, quizartinib has, to date, been dosed as a liquid oral treatment. We have recently developed a solid dosage form (tablet) of quizartinib and successfully completed a Phase 1 clinical trial in healthy volunteers to confirm the relative bioavailability between the liquid and the tablet forms. We anticipate incorporating the tablet in future clinical development, including our

Table of Contents

planned Phase 3 clinical trial, subject to guidance from the FDA. We may encounter delays in the manufacture of this tablet form, in which case we would need to continue to use the liquid form in future trials. In any event, if approved, our commercial strategy is to have both the tablet form and liquid forms in order to address the needs of multiple patient populations.

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

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

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

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

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

We received Fast Track designation for our drug candidate quizartinib for the treatment of patients 60 years of age or older with FLT3-ITD positive AML in first relapse or refractory to first line chemotherapy and treatment of patients 18 years or older with FLT3-ITD positive AML in second relapse or refractory to second line salvage therapy. Fast track designation is a process designed to facilitate the development and expedite the review of new drugs intended to treat serious or life-threatening diseases or conditions and that have the potential to address an unmet medical need for such disease or condition. Fast Track designation applies to the product and the specific indication for which it is being studied. Once a Fast Track designation is obtained, the FDA may consider for review on a rolling basis sections of the NDA before the complete application is submitted if the applicant provides and the FDA approves a schedule for the submission of the sections of the NDA and the applicant pays applicable user fees upon submission of the first section of the NDA. However, the time period specified in the Prescription Drug User Fee Act, which governs the time period goals the FDA has committed to reviewing an application, does not begin until the complete application is accepted for filing. Although we received Fast Track designation for quizartinib, the FDA may later decide that quizartinib no longer meets the conditions for qualification. In addition, Fast Track designation may not provide us with a material commercial advantage.

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

We currently do not have a commercial organization for the marketing, sales and distribution of pharmaceutical products. In order to commercialize any products, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services.

Table of Contents

We contemplate establishing (either internally or through a contract sales force) our own commercial capabilities to market, sell and distribute quizartinib, if approved, in North America and plan to partner with third parties to commercialize quizartinib in other markets.

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

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

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

We cannot be certain that our drug candidates will produce commercially viable drugs that safely and effectively treat cancer or other diseases. To date, our technology platform has yielded only a small number of drug candidates other than quizartinib. In addition, we have limited preclinical and clinical data with respect to any of these other potential drug candidates. Even if we are successful in completing preclinical and clinical development and receiving regulatory approval for one commercially viable drug for the treatment of one disease, we cannot be certain that we will also be able to develop and receive regulatory approval for other drug candidates for the treatment of other forms of that disease or other diseases. If we fail to develop and commercialize viable drugs, we will not be successful in developing a pipeline of potential drug candidates to follow quizartinib, and our business prospects would be harmed significantly.

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

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

- the efficacy and safety of such drug candidates as demonstrated in clinical trials;
- the clinical indications for which the drug candidate is approved;
- acceptance by physicians, major operators of cancer clinics and patients of the drug as a safe and effective treatment;
- the potential and perceived advantages of drug candidates over alternative treatments;
- the safety of drug candidates seen in a broader patient group, including its use outside the approved indications;

36

Table of Contents

the prevalence and severity of any side effects;

product labeling or product insert requirements of the FDA or other regulatory authorities;

the timing of market introduction of our products as well as competitive products;

the cost of treatment in relation to alternative treatments;

the availability of adequate reimbursement and pricing by third party payors and government authorities;

relative convenience and ease of administration; and

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

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

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

The biotechnology and pharmaceutical industries are intensely competitive. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis drug products that are more effective or less costly than quizartinib or any drug candidate that we are currently developing or that we may develop.

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

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

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

discover and develop highly selective and potent small molecule drugs that inhibit validated kinase targets and that are superior to other products in the market;

attract qualified scientific, product development and commercial personnel;

obtain patent and/or other proprietary protection for our medicines and technologies;

obtain required regulatory approvals; and

successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

Table of Contents

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

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

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

We intend to seek approval to market quizartinib in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for quizartinib or any of our other drug candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of prescription pharmaceuticals and biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug candidate. In addition, market acceptance and sales of our drug candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for any of our drug candidates and may be affected by existing and future health care reform measures.

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

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

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

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

government s role in the U.S. healthcare industry may further lower rates of reimbursement for pharmaceutical products.

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

Table of Contents

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

- the demand for any drug candidates for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenues and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

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

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

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

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

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

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

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with all of our employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. The loss of the services of any of our

Table of Contents

executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations. We do not maintain key man insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

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

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

As of June 30, 2013, we employed 48 employees, 43 of whom were full-time. As our development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional managerial, operational, clinical, regulatory, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

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

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

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

Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors and consultants and collaborators are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties to manufacture our drug candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our drug candidates could be delayed.

40

Table of Contents

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

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

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

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

- differing regulatory requirements for drug approvals in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;

production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and

business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

Table of Contents

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

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

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

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

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

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

- decreased demand for our drug candidates or products that we may develop;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;

a diversion of management s time and our resources;

substantial monetary awards to trial participants or patients;

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

loss of revenue;

Table of Contents

exhaustion of any available insurance and our capital resources;

the inability to commercialize our drug candidates; and

a decline in our share price.

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

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

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

Risks Related to Our Financial Position and Capital Requirements

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

Our operations began in 2000 and we have only a limited operating history upon which you can evaluate our business and prospects. Our operations to date have been limited to conducting product development activities for quizartinib and other drug candidates and performing research and development with respect to our clinical and preclinical programs. In addition, as an early stage company, we have limited experience and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical area. Nor have we demonstrated an ability to obtain regulatory approval for or to commercialize a drug candidate. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing pharmaceutical products.

We have incurred significant operating losses since our inception, including consolidated net losses of $3.1 million and $15.3 million for the six months ended June 30, 2013 and 2012, respectively. As of June 30, 2013, we had an accumulated deficit of $240.1 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders equity and working capital. Our losses have resulted principally from costs incurred in our discovery and development activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our discovery, research,

Table of Contents

development and commercialization activities, including the clinical development and planned commercialization of our lead drug candidate, quizartinib, and incur the additional costs of operating as a public company. In addition, if we obtain regulatory approval of quizartinib, we may incur significant sales and marketing expenses. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

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

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

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

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

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

On March 31, 2010, we entered into a $12.0 million venture loan and security agreement, or the Venture Loan, with Compass Horizon Funding Company LLC and Oxford Finance Corporation, collectively, the Lenders. The Venture Loan is secured by a lien covering substantially all of our assets, excluding intellectual property, and we also pledged as collateral a portion of our equity interests in our subsidiaries. The Venture Loan is amortizing and we are obligated to make monthly payments of principal and interest through the maturity date of October 1, 2013, assuming there is no default that results in acceleration of the debt. As of June 30, 2013, the outstanding principal balance of the Venture Loan was $1.8 million.

The loan agreement governing the Venture Loan contains customary affirmative and negative covenants and events of default. The affirmative covenants include, among others, covenants requiring us to maintain our legal existence and governmental approvals, deliver certain financial reports and maintain insurance coverage. The negative covenants include, among others, restrictions on transferring collateral, changing our business, incurring additional indebtedness, engaging in mergers or acquisitions, paying dividends or making other distributions, making investments and creating other liens on our assets, in each case subject to customary exceptions. If we default under the Venture Loan, the

Lenders may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Further, if we are liquidated, the Lenders right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. The Lenders could declare a default

Table of Contents

under the Venture Loan upon the occurrence of any event that they interpret as a material adverse change as defined under the loan agreement, thereby requiring us to repay the loan immediately or to attempt to reverse the declaration of default through negotiation or litigation. Any declaration by the Lenders of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

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

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

- continue the clinical development of quizartinib in AML, including our ongoing Phase 2b clinical trials and planned Phase 3 clinical trial, as well as the preclinical and clinical development of other drug candidates;
- prepare regulatory submissions for regulatory approval of quizartinib;
- continue our research and development programs to advance our internal product pipeline;
- launch and commercialize quizartinib and any other drug candidates for which we receive regulatory approval, including building our own commercial capabilities to sell, market, and distribute quizartinib in North America; and
- service and/or repay the Venture Loan.

We believe our existing cash and cash equivalents will be sufficient to fund our operations through at least the next 12 months. In particular, we believe our existing cash and cash equivalents, along with interest thereon, will be sufficient to fund such development through receipt of topline data from our planned Phase 3 clinical trial in patients with relapsed/refractory AML. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We will require additional capital for the further development and commercialization of quizartinib and our other drug candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

We cannot be certain that additional funding will be available on acceptable terms, or at all. Subject to limited exceptions, the Venture Loan prohibits us from incurring additional indebtedness. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our drug candidates or other research and development initiatives. We also could be required to:

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

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

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

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

Table of Contents

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

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an ownership change (generally defined as a greater than 50% change (by value) in its equity ownership over a three year period), the corporation s ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We believe that, as a result of our IPO, the concurrent private placement and other transactions that have occurred over the past three years, we have experienced an ownership change. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As of December 31, 2012, we had federal and state net operating loss carryforwards of approximately $151.2 million and $143.1 million, respectively, and federal research and development credits of $5.0 million which could be limited if we experience an ownership change.

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

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

At June 30, 2013, we had $85.3 million of cash and cash equivalents. While we are not aware of any downgrades, material losses, or other significant deterioration in the fair value of our cash equivalents since June 30, 2013, no assurance can be given that further deterioration of the global credit and financial markets would not negatively impact our current portfolio of cash equivalents or marketable securities or our ability to meet our financing objectives. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

Risks Related to Our Intellectual Property

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

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

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

Table of Contents

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

In addition to the protection afforded by patents, we seek to rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. Although we require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

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

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

Table of Contents

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

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our drug candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our drug candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our drug candidates, which could harm our business significantly.

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

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

Interference proceedings provoked by third parties or brought by the U.S. PTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our collaborators or licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or interference proceedings may fail and, even if successful, may result in

Table of Contents

substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

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

Periodic maintenance fees on any issued patent are due to be paid to the U.S. PTO and foreign patent agencies in several stages over the lifetime of the patent. The U.S. PTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

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

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

Risks Related to Ownership of our Common Stock

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

Prior to our IPO, there was no public market for our common stock. Since our IPO in May 2013, the trading price of our common stock has ranged from a low of approximately $6 to a high of approximately $17. The trading price of our common stock is likely to continue to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this Risk Factors section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our planned Phase 3 clinical trial of quizartinib or any ongoing or future clinical trials we may conduct, or changes in the development status of quizartinib or any other drug candidate;
- any delay in filing our NDA for quizartinib and any adverse development or perceived adverse development with respect to the FDA s review of the NDA, including without limitation the FDA s issuance of a refusal to file letter or a request for additional information;
- adverse results or delays in clinical trials;

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

Table of Contents

adverse regulatory decisions, including failure to receive regulatory approval for quizartinib or the companion diagnostic;

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

adverse developments concerning our collaborations and our manufacturers;

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

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

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

additions or departures of key scientific or management personnel;

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

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

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

our ability to effectively manage our growth;

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

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

actual or anticipated variations in quarterly operating results;

our cash position;

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

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

changes in the market valuations of similar companies;

overall performance of the equity markets;

issuances of debt or equity securities;

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

trading volume of our common stock;

changes in accounting practices;

ineffectiveness of our internal controls;

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

significant lawsuits, including patent or stockholder litigation;

general political and economic conditions; and

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

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

Table of Contents

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

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

As of June 30, 2013, our executive officers, directors, 5% stockholders and their affiliates owned approximately 72% of our voting stock. Therefore, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

Table of Contents

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

As a public company, we have incurred, and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC, and the Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and proxy access. Recent legislation permits smaller emerging growth companies to implement many of these requirements over a longer period and up to five years from the IPO. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

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

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

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in our prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of June 30, 2013, we have outstanding a total of 17,876,704 shares of common stock. Of these shares, only the 8,125,000 shares of common stock sold in our IPO are freely tradable without restriction in the public market. Citigroup Global Markets Inc. and Leerink Swann LLC, however, may, in their sole discretion, permit our officers, directors and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

We expect that the lock-up agreements pertaining to the IPO will expire 180 days from the date of our prospectus, May 15, 2013. After the lock-up agreements expire, up to an additional 9,587,558 shares of common stock will be eligible for sale in the public market, 5,215,402 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. In addition, 1,970,329 shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

The holders of 5,215,402 shares of our common stock, or approximately 29% of our total outstanding common stock as of June 30, 2013, will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Table of Contents

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

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

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

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

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

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

Table of Contents

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

Use of Proceeds

On May 15, 2013, we commenced our IPO pursuant to a registration statement on Form S-1 (File No. 333-186760) that was declared effective by the SEC on May 15, 2013 and that registered an aggregate of 9,343,750 shares of our common stock for sale to the public at a price of $8.00 per share at an aggregate offering price of $74,750,000. On May 21, 2013, we sold 8,125,000 shares of our common stock to the public at a price of $8.00 per share for an aggregate gross offering price of $65,000,000. The offering has now terminated and consequently we may not sell under that registration statement the 1,218,750 remaining shares of common stock. Citigroup Global Markets Inc. and Leerink Swann LLC acted as joint booking-running managers for the offering, and BMO Capital Markets Corp. and Robert W. Baird & Co. Incorporated served as co-managers for the offering.

The underwriting discounts and commissions in connection with the offering totaled approximately $4.6 million. We incurred additional costs of approximately $2.3 million in offering expenses, which when added to the underwriting discounts and commissions paid by us, amounts to total fees and costs of approximately $6.9 million. Thus, the net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, were approximately $58.1 million. No offering costs were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

Upon receipt, the net proceeds from our IPO were invested in cash equivalents. As of June 30, 2013, we estimate that we had used approximately $19,000 for the purchase of equipment and approximately $6.3 million for working capital expenditures. We intend to use the remainder of the net proceeds from the IPO to fund the continued clinical development of quizartinib, to fund the continued development of our other programs and for working capital and other general corporate purposes. The amounts and timing of our actual expenditures depend on numerous factors, including the ongoing status of and results from clinical trials and other studies, as well as any strategic partnerships that we may enter into with third parties for our drug candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from our IPO and the concurrent private placement and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our stock.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosure

Not applicable.

Item 5. Other Information

None.

Table of Contents

Item 6. Exhibits

Exhibit Number	Description of Document
3.1(1)	Amended and Restated Certificate of Incorporation.
3.2(1)	Amended and Restated Bylaws.
4.1(2)	Form of Common Stock Certificate.
4.2(2)	Form of Warrant to Purchase Common Stock issued by the Registrant to June, July and December 2009 bridge financing investors.
4.3(2)	Form of Warrant to Purchase Common Stock issued by the Registrant to September 2010 bridge financing investors.
4.4(2)	Warrant issued by the Registrant on October 5, 2005 to Oxford Finance Corporation.
4.5(2)	Warrant issued by the Registrant on December 22, 2005 to Oxford Finance Corporation.
4.6(2)	Form of Warrant issued by the Registrant to Oxford Finance Corporation pursuant to 2006 Master Security Agreement.
4.7(2)	Form of Warrant issued by the Registrant to Webster Bank, National Association pursuant to 2006 Master Security Agreement.
4.8(2)	Warrant issued by the Registrant on October 6, 2005 to Horizon Technology Funding Company II, LLC.
4.9(2)	Warrant issued by the Registrant on October 6, 2005 to Horizon Technology Funding Company III, LLC.
4.10(2)	Warrant issued by the Registrant on September 24, 2007 to Horizon Technology Funding Company V, LLC.
4.11(2)	Warrant issued by the Registrant on March 31, 2010 to Compass Horizon Funding Company LLC.
4.12(2)	Warrant issued by the Registrant on March 31, 2010 to Oxford Finance Corporation.
4.13(2)	Form of Warrant to Purchase Series D-2 Preferred Stock issued by the Registrant to May 2011 Series D-2 preferred stock financing investors.
4.14(2)	Termination and Warrant Amendment Agreement dated May 18, 2012 among the Registrant and certain holders of Series D-2 preferred stock warrants.
4.15(2)	Second Warrant Amendment Agreement dated October 25, 2012 among the Registrant and certain holders of Series D-2 Preferred stock warrants.
4.16(2)	Form of Warrant to Purchase Common Stock issued by the Registrant to October 2012 Series E preferred stock financing investors.
4.17(2)	Sixth Amended and Restated Investors Rights Agreement dated October 25, 2012 among the Registrant and certain of its stockholders, as amended.
4.18	Termination and Amendment Agreement dated May 15, 2013 among the Registrant and certain of its stockholders.
10.1+(2)	2011 Amended and Restated Equity Incentive Plan and forms of Stock Option Agreement thereunder, as amended.
10.2+(2)	2013 Equity Incentive Plan and form of Stock Option Agreement thereunder.
10.3+(2)	2013 Employee Stock Purchase Plan.
10.4+(2)	Non-employee Director Compensation Policy.
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15-d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended

Table of Contents

Exhibit Number	Description of Document
32.1	Certification of Principal Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

+ Indicates management contract or compensatory plan.
(1) Incorporated by reference to the Registrant s Current Report on Form 8-K, filed with the SEC on May 21, 2013.
(2) Incorporated by reference to the Registrant s Registration Statement on Form S-1, as amended (File No. 333-186760), originally filed with the SEC on February 20, 2013.

Table of Contents

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Ambit Biosciences Corporation

Date: August 13, 2013

By: /S/ MICHAEL A. MARTINO
Michael A. Martino

President, Chief Executive Officer and Director

(on behalf of the registrant and as the registrant s Principal Executive Officer)

By: /S/ ALAN FUHRMAN
Alan Fuhrman

Chief Financial Officer

(on behalf of the registrant and as the registrant s Principal Financial and Accounting Officer)