Mirati Therapeutics, Inc.
Form 10-Q
November 12, 2013
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2013

or

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

Commission File Number: 001-35921

MIRATI THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**46-2693615**
(State of Incorporation)	(I.R.S. Employer Identification No.)
9363 Towne Centre Drive, Suite 200 **San Diego, California**	**92121**
(Address of Principal Executive Offices)	(Zip Code)

(858) 332-3410

(Registrant s Telephone Number, Including Area Code)

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 such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x No o

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 during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No o

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 Securities Exchange Act of 1934.

Large accelerated filer	o	Accelerated filer	o
Non-accelerated filer	o (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 o No x

Total shares of common stock outstanding as of the close of business on November 7, 2013:

Class	**Number of Shares Outstanding**
Common Stock, $0.001 par value	13,261,862

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MIRATI THERAPEUTICS, INC.

FORM 10-Q

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PART I. FINANCIAL INFORMATION

ITEM 1. ***Financial Statements***

MIRATI THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS

(In thousands)

(Unaudited)

	September 30, 2013	December 31, 2012
ASSETS		
Current assets		
Cash and cash equivalents	**$ 7,616**	$ 18,403
Marketable securities	**7,418**	18,580
Restricted cash equivalents and marketable securities	**291**	302
Interest and other receivables	**186**	507
Other current assets	**1,510**	1,537
Total current assets	**17,021**	39,329
Security deposits	**99**	67
Restricted cash equivalents and marketable securities	**80**	72
Deferred costs	**374**	
Property and equipment, net	**427**	333
Total assets	**$ 18,001**	$ 39,801
LIABILITIES AND STOCKHOLDERS EQUITY (DEFICIT)		
Current liabilities		
Accounts payable and accrued liabilities	**6,555**	5,272
Current portion of other liability	**60**	68
Share-based compensation liability	**2,144**	
Warrant liability	**32,803**	
Total current liabilities	**41,562**	5,340
Other liability		45
Total liabilities	**41, 562**	5,385
Stockholders equity (deficit)		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; none issued and outstanding at both September 30, 2013 and December 31, 2012		
Common stock, $0.001 par value; 100,000,000 authorized; 9,960,621 and 9,957,725 issued and outstanding at both September 30, 2013 and December 31, 2012	**10**	10
Warrants		11,153
Additional paid-in capital	**154,069**	154,224
Accumulated other comprehensive income	**9,520**	9,520

Accumulated deficit		**(187,160)**		(140,491)
Total stockholders equity (deficit)		**(23,561)**		34,416
Total liabilities and stockholders equity (deficit)	**$**	**18,001**	**$**	39,801

See accompanying notes

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MIRATI THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands except for share and per share amounts)

(Unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2013	**2012**	**2013**	**2012**
Expenses				
Research and development, net	$ **5,492**	$ 4,249	**$ 15,477**	$ 10,105
General and administrative	**3,710**	1,717	**8,616**	4,019
Total operating expenses	**9, 202**	5,966	**24,093**	14,124
Loss from operations	**(9,202)**	(5,966)	**(24,093)**	(14,124)
Other (expense) income, net	**(20,141)**	34	**(17,418)**	172
Loss before income taxes	**(29,343)**	(5,932)	**(41,511)**	(13,952)
Income tax expense	**55**	14	**115**	27
Net loss and comprehensive loss	$ **(29,398)**	$ (5,946)	**$ (41,626)**	$ (13,979)
Basic and diluted net loss per share	$ **(2.95)**	$ (0.93)	**$ (4.18)**	$ (2.20)
Weighted average number of shares used in computing net loss per share, basic and diluted	**9,957,896**	6,361,093	**9,957,792**	6,359,216

See accompanying notes

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MIRATI THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

(Unaudited)

	Nine months ended September 30,	
	2013	**2012**
Operating activities		
Net loss and comprehensive loss	$ **(41,626)**	$ (13,979)
Non-cash adjustments reconciling net loss to operating cash flows		
Depreciation of property and equipment	**105**	98
Share-based compensation expense	**1,094**	1,398
Change in fair value of warrant liability	**16,609**	
Change in fair value of share-based compensation liability	**871**	
Lease incentive liability	**(53)**	64
Changes in operating assets and liabilities		
Interest and other receivables	**321**	61
Other current assets	**27**	(105)
Deferred costs	**(374)**	
Accounts payable and accrued liabilities	**1,283**	1,001
Cash flows used for operating activities	**(21,743)**	(11,462)
Investing activities		
Purchases of property and equipment	**(199)**	(69)
Purchases of marketable securities	**(25,725)**	(17,325)
Security deposit	**(32)**	(12)
Restricted cash equivalents and marketable securities	**3**	(17)
Disposal and maturities of marketable securities	**36,887**	24,363
Cash flows provided by investing activities	**10,934**	6,940
Financing activities		
Exercise of warrants	**22**	
Costs of reorganization		(16)
Cash flows used for financing activities	**22**	(16)
Decrease in cash and cash equivalents	**(10,787)**	(4,538)
Effect of exchange rate changes on cash and cash equivalents		299
Cash and cash equivalents, beginning of period	**18,403**	9,882
Cash and cash equivalents, end of period	**$ 7,616**	$ 5,643

See accompanying notes

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MIRATI THERAPEUTICS, INC.

Notes to Consolidated Financial Statements

September 30, 2013

(Unaudited)

1. DESCRIPTION OF BUSINESS

Mirati Therapeutics, Inc. (Mirati or the Company) is a clinical stage biopharmaceutical company focused on developing a pipeline of targeted oncology products.

The Company has a wholly owned subsidiary in Canada, MethylGene, Inc. (MethylGene). MethylGene s common stock was listed on the Toronto Stock Exchange from June 29, 2004 until July 26, 2013 under the ticker symbol MYG . The Company also has an indirect, wholly-owned subsidiary, MethylGene US Inc., which was incorporated in Princeton, New Jersey on December 20, 2011 and started business activity in 2012. The Company s common stock has been listed on the NASDAQ Capital Market since July 15, 2013 under the ticker symbol MRTX . The Company is a holding company with minimal assets other than the stock of its subsidiary in Canada, MethylGene Inc., and primarily conducts its operations through MethylGene and MethylGene US Inc. Refer to Note 2 under the heading Basis of Presentation for further discussion of the Company s corporate structure.

2. BASIS OF PRESENTATION

The information contained herein has been prepared in accordance with instructions for Form 10-Q and Article 10 of Regulation S-X. The information as of September 30, 2013, and for the nine months ended September 30, 2013 and 2012, is unaudited. In the opinion of management, the information reflects all adjustments necessary to make the results of operations for the interim periods a fair statement of such operations. All such adjustments are of a normal recurring nature. Interim results are not necessarily indicative of results for the full year. The consolidated balance sheet at December 31, 2012 has been derived from the audited consolidated financial statements at that date, but does not include all information and footnotes required by U.S. generally accepted accounting principles for complete financial statements. For more complete financial information, these financial statements should be read in conjunction with the audited consolidated financial statements included in Mirati s Registration Statement on Form 10 (No. 001-35921), originally filed with the Securities and Exchange Commission (SEC) on May 10, 2013, as amended.

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. The Company was created to enter into an arrangement agreement with MethylGene described below.

On May 8, 2013, the Company s Board of Directors approved and the Company entered into an arrangement agreement with MethylGene. Subject to the terms and conditions of the arrangement agreement, which was consummated on June 28, 2013, the shareholders of MethylGene received one share of the Company s common stock in exchange for every 50 common shares of MethylGene, which had the effect of a 50 for 1

reverse split of MethylGene s common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. Such transaction is referred to herein as the Arrangement . In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of the Company s common stock, and a proportionate adjustment was made to the exercise price. Upon completion of the Arrangement, MethylGene became the Company s wholly-owned subsidiary. The shares of the Company s common stock issued at the closing of the Arrangement were issued in reliance upon the exemption from registration under Section 3(A)(10) of the Securities Act of 1933, as amended.

These financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities in the normal course of business. The Company has incurred significant operating losses since inception and has relied on its ability to fund its operations through private and public equity financings and a debt financing. At September 30, 2013, the Company had $15.4 million in cash, cash equivalents, marketable securities and restricted cash.

As described in Note 14, Subsequent Events, the Company completed a public offering of its common stock (the Offering) on October 29, 2013 for net proceeds of $53.0 million, after deducting underwriting discounts and commissions of $3.4 million and other estimated offering expenses of $0.5 million payable by us. The Company expects that its current cash, cash equivalents and marketable securities, together with the net proceeds from the Offering, will sustain its operations through the end of 2015.

These condensed interim consolidated financial statements are presented in U.S. dollars, which effective January 1, 2013, is also the functional currency of the Company.

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3. SIGNIFICANT ACCOUNTING POLICIES

Foreign Currency Transactions

Foreign currency transactions are initially recorded by the Company using the exchange rates prevailing at the date of the transaction. At the balance sheet date, monetary assets and liabilities denominated in foreign currencies are translated at the period-end rates of exchange. Non-monetary assets and liabilities are translated at the historical exchange rates. Exchange gains and losses arising from the translation of foreign currency items are included in other (expense) income in the consolidated statements of operations and comprehensive loss. The Company recognized net foreign exchange gains of $66,000 and net foreign exchange losses of $15,000 in other (expense)/income in the consolidated statement of operations and comprehensive loss for the three months ended September 30, 2013 and 2012, respectively. The Company recognized net foreign exchange losses of $593,000 and $4,000 in other (expense) income in the consolidated statement of operations and comprehensive loss for the nine months ended September 30, 2013 and 2012, respectively.

Reclassification of Share-Based Compensation Liability

MethylGene had granted stock options denominated in Canadian dollars under the 1997 Equity Plan to Canadian and United States, or US, based employees and directors until July 26, 2013. Following the delisting of the Company s shares from the Toronto Stock Exchange, the options denominated in Canadian dollars that were granted to US-based employees and US-based directors are subject to liability accounting, in accordance with Accounting Standards Codification, or ASC, 718, *Compensation-Stock Compensation* . The Company revalued such options as of July 26, 2013 and recorded a share-based compensation liability of $1.1 million with a corresponding reduction in additional paid-in capital.

At each reporting period subsequent to July 26, 2013, the Company will adjust the fair value of the share-based compensation liability and any corresponding increase or decrease to the liability will be recorded as equity reclassification adjustment or stock compensation expense on the consolidated statement of operations and comprehensive loss. The estimated fair value is determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining life of the options, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock. As of September 30, 2013, the fair value of the liability was $2.1 million resulting in a reduction in additional paid-in capital of $1.2 million and an increase in stock compensation expense of $0.9 million for the three and nine months ended September 30, 2013 in the consolidated statement of operations and comprehensive loss.

Reclassification of Warrants

In 2011 and 2012, the Company issued common stock warrants in connection with the issuance of common stock through private placements (referred to as the 2011 Warrants and the 2012 Warrants). The exercise prices of the 2011 and 2012 Warrants were denominated in Canadian dollars. Upon the issuance of the 2011 and 2012 Warrants, the Company allocated the net proceeds to common stock and warrants based on their relative fair values, and calculated the fair value of the issued common stock warrants utilizing the Black-Scholes option-pricing model. The allocated fair value was then recorded as warrants within stockholders equity on the consolidated balance sheet. The fair value was not recalculated in periods subsequent to the date of issuance.

The change in its functional currency to the U.S. dollar effective January 1, 2013 changed how the Company accounts for its warrants which have exercise prices denominated in Canadian dollars. Upon the change in functional currency, the Company classified these warrants as a current liability and recorded a warrant liability of $16.2 million which represents the fair market value of the warrants at that date in accordance with Accounting Standards Codification, or ASC, 815, *Derivatives and Hedging*. The initial fair value recorded as warrants within stockholders equity of $11.2 million was reversed. The change in fair value related to periods prior to January 1, 2013 of $5.0 million was recorded as an adjustment to accumulated deficit. At each reporting period subsequent to January 1, 2013, the Company will adjust the fair value of the warrant liability and any corresponding increase or decrease to the warrant liability will be recorded as a component of other (expense) income on the consolidated statement of operations and comprehensive loss. The estimated fair value is determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining contractual term of the warrants, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock. The fair value of the warrant liability was $32.8 million at September 30, 2013 and the Company recorded a loss of $20.6 million for the three months ended September 30, 2013 and a loss of $16.6 million for the nine months ended September 30, 2013 which is included in other (expense) income in the consolidated statement of operations and comprehensive loss.

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4. COLLABORATION AGREEMENTS

Collaboration with Taiho

In October 2003, the Company entered into a license and research and development collaboration agreement with Taiho, a leading Japanese specialty oncology company, for mocetinostat and our small molecule HDAC inhibitor program for oncology for Japan, South Korea, Taiwan and China, or collectively the Taiho Territory. Under the terms of the agreement, the Company received an up-front license fee, equity investment and a contract research payment of $3.8 million. In addition, the Company may receive milestone payments based on successful development, regulatory approval, and commercialization of an HDAC oncology product totaling up to $16.2 million. The Company may also receive royalty payments in connection with commercial sales of HDAC oncology products as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales. Such royalties may be reduced, subject to a mid-single digit floor, by (i) credits against recoupable development costs paid by Taiho to the Company and/or (ii) reduction by a percentage in the range of 20-30% in the event a generic competitor is introduced in a particular market, other than in China. Taiho provided the Company with contract research payments for scientists for two years at $2.0 million per year as well as funding for contract preclinical and contract clinical development costs in North America for mocetinostat, which totaled, in the aggregate, $5.4 million. In total, the Company has received $15.0 million from Taiho under the agreement, including a $1.5 million milestone payment relating to the start of the first Phase 2 trial with mocetinostat. However, upon the execution of the Company s agreement with Celgene in 2008, Taiho s funding obligations for clinical trials in North America ceased. In addition, Taiho s collaboration entailed in-kind support in their research laboratories in order to select a next generation compound, and in some cases, will support a portion of preclinical development costs in North America. Currently, there are no efforts by either (i) Taiho to further advance mocetinostat in the Taiho Territory or (ii) Taiho or the Company to further advance other small molecule HDAC inhibitors that would be covered by this agreement. However, Taiho has retained rights in the Taiho Territory to certain sirtuin inhibitors for cancer. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or ten years after the first commercial sale in Japan. Additionally, Taiho has a unilateral right to terminate the agreement for any reason with 30 days written notice, and the Company has a unilateral right to terminate the agreement if Taiho fails to make an undisputed payment. An arbitrator may terminate the agreement for a breach of obligations if such breach has remained uncured for 90 days. As long as the agreement continues, the Company is obligated to use reasonable efforts to contract with Taiho for its supply of the active bulk compounds for the sale of mocetinostat outside of the Taiho Territory. In the event the parties wish to collaborate on the development of another HDAC inhibitor covered by this agreement or sirtuin inhibitor retained by Taiho, Taiho would be obligated to contribute to preclinical and clinical costs of such a compound. Such a compound would also be subject to potential development milestones and royalties. The Company is in preliminary discussions with Taiho to consider whether any amendments to the agreement should be made based upon its development plans for mocetinostat and their rights under the agreement.

Collaboration with Otsuka

In March 2008, the Company entered into a worldwide research collaboration and license agreement with Otsuka, a global Japanese pharmaceutical company, for the development of novel, small molecule, kinase inhibitors for local delivery and treatment of ocular diseases, excluding cancer. The Company was responsible for the design, characterization and initial screening of kinase inhibitors and control over determining which compounds to synthesize. Otsuka was responsible for funding efficacy and toxicity studies, as well as preclinical and clinical development of compounds. Otsuka is also responsible for the global commercialization of any resulting product. Under the terms of the agreement, the Company received an up-front license fee of $2.0 million. The Company may receive additional payments based on successful development, regulatory, commercialization and sales milestones that could total up to $50.5 million. The Company may also receive royalty payments in connection with commercial sales of licensed products under the agreement as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales, subject to reduction by a percentage in the range of 40-50% in the event a generic competitor is introduced in a given market or intellectual property protection in a particular market does not exist or expires in a given market. The Company may receive aggregate milestone payments of up to $50.5 million under this agreement as follows: $7.5 million relates to development activities, $22.0 million relates to the completion of regulatory approvals and $21.0 million relates to the achievement of certain sale goals. Otsuka provided $1.9 million in research funding for the initial 18 months of

the research collaboration, which was extended on three occasions: September, 2009; April 2010 and June 2010. The research component of the agreement ended on June 30, 2011. The Company received a total of $4.5 million in research funding from the research component of this agreement. In October 2009, Otsuka made, in connection to the terms

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of the agreement, a $1.5 million equity investment in the Company s shares of common stock at a share price of CND$21.30 (or US$20.27, as converted), which was a 20% premium over the five-day volume-weighted average closing price at the date of the transaction. On June 30, 2010, the collaboration agreement was amended to, among certain other changes, provide Otsuka the rights to synthesize a limited number of compounds predetermined by the Company. A lead molecule was selected in June 2011 for further development. The research portion of the collaboration between the Company and Otsuka concluded on June 30, 2011; however, the term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or if no patent has issued in such country, then 12 years after the first sale of a licensed product by Otsuka. Otsuka has a unilateral right to terminate the agreement for any reason with 90 days written notice and either party may terminate the agreement for a breach of obligations of the other party if such breach has remained uncured for 120 days (or 30 days for a breach of payment). Otsuka is currently advancing the lead compound through late preclinical development.

Collaboration with EnVivo

In March 2004, the Company entered into a proof of concept and option agreement with EnVivo, a private U.S. biotechnology company focusing on the treatment and prevention of certain neurodegenerative diseases, to exploit the Company s HDAC inhibitors in diseases such as Huntington s disease, Parkinson s disease and Alzheimer s disease. In February 2005 the Company signed an exclusive research, collaboration and license agreement. Over the course of 2005, EnVivo paid the Company $0.6 million for research, plus a $0.5 million license fee, for a total of $1.1 million. As part of this agreement, EnVivo received a warrant to purchase 1,050 shares of common stock at an exercise price of CND$214.30 (or US$203.76, as converted). The warrant expired in March 2007. In February 2008, the Company exercised its right to opt-out of the program. As a result, the Company granted EnVivo exclusive rights to our HDAC inhibitors for neurodegenerative diseases and the Company ceased research and development funding for this program. The Company is prohibited under the surviving terms of the agreement with EnVivo from developing or commercializing any HDAC products in the field of certain neurodegenerative diseases, including Huntington s disease, Parkinson s disease and Alzheimer s disease. The Company may receive royalty payments in an aggregate amount equal to a single digit percentage of net sales of any approved compound and will share in any sublicense income from future partnerships that EnVivo may enter into.

5. CASH AND CASH EQUIVALENTS

(in thousands)	**September 30, 2013**	**December 31, 2012**
Cash at bank and on hand	**$ 2,958**	$ 2,823
Bankers acceptances	**1,262**	1,369
Treasury bills		5,026
Promissory notes		6,020
Commercial papers	**2,716**	753
Term deposit notes	**971**	2,714
	7,907	18,705
Less: restricted cash equivalents	**(291)**	(302)
	7,616	$ 18,403

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6. MARKETABLE SECURITIES

(in thousands)	September 30, 2013	December 31, 2012
Bankers acceptances issued in Canadian currency, earning interest at a rate of 1.06% (1.20% in 2012) and maturing on November 1, 2013 (February 19, 2013 in 2012)	**$ 80**	$ 72
Commercial paper issued in Canadian currency, earning interest at rates ranging from 1.01% to 1.12% and maturing on various dates from February 21, 2013 to May 14, 2013		5,026
Guaranteed investment certificates issued in Canadian currency, earning interest at rates ranging from 1.20% to 1.30% (1.15% to 1.35% in 2012) and maturing on various dates from November 12, 2013 to September 29, 2014 (January 7, 2013 to September 16, 2013 in 2012)	**7,418**	6,518
Term deposits issued in Canadian currency, earning interest at rates ranging from 1.30% to 1.33% and maturing on various dates from March 18, 2013 to April 15, 2013		7,036
	7,498	18,652
Less restricted marketable securities	**(80)**	(72)
	$ **7,418**	$ 18,580

7. INTEREST AND OTHER RECEIVABLES

(in thousands)	September 30, 2013	December 31, 2012
Other receivables	**$ 160**	$ 425
Interest receivable	**26**	82
	$ 186	$ 507

8. OTHER CURRENT ASSETS

(in thousands)	September 30, 2013	December 31, 2012
Refundable research and development tax credits	**$ 654**	$ 593
Commodity taxes	**355**	165
Prepaid expenses	**501**	779
	$ 1,510	$ 1,537

9. ACCOUNTS PAYABLE AND ACCRUED LIABILITIES

(in thousands)	September 30, 2013	December 31, 2012
Accounts payable	**$ 2,001**	$ 1,752
Accrued compensation and benefits	**1,156**	834
Accrued expenses	**3,398**	2,686
	$ 6,555	$ 5,272

10. INVESTMENT TAX CREDITS

The Company recorded $196,000 and $678,000 related to refundable investment tax credits as a reduction of research and development expenses for the three-month period and nine-month period ended September 30, 2013, respectively, and $136,000 and $1.5 million for the three-month period and nine-month period ended September 30, 2012, respectively.

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11. NET LOSS PER SHARE

Basic and diluted

Net loss per share is calculated by dividing the net loss of the Company by the weighted average number of shares of common stock outstanding during the year. The following table presents potentially dilutive securities not included in the calculation of diluted net loss per share because to do so would be anti-dilutive:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Common stock options	51,393	28,904	1,436	12,755
Common stock warrants	481,765	771,684	92,192	780,034
Total	533,158	800,588	93,628	792,789

12. FAIR VALUE MEASUREMENT AND FINANCIAL INSTRUMENTS

The following tables present information about the Company s assets and liabilities that are measured at fair value on a recurring basis for the periods presented and indicates the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value.

In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities.

Fair values determined by Level 2 inputs utilize data points that are observable such as quoted prices, interest rates and yield curves.

Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability.

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There were no transfers in or out of Level 1, Level 2 or Level 3 measurements for the periods presented (in thousands):

	September 30, 2013	**Level 1**	**Level 2**	**Level 3**
Assets				
Cash equivalents	**$ 4,657**		**$ 4,657**	**$**
Marketable securities	**7,418**		**7,418**	
Restricted cash equivalents and marketable securities	**371**		**371**	
	$ 12,446	**$**	**$ 12,446**	**$**
Liability				
Share-based compensation liability	**2,144**			**2,144**
Warrant liability	**32,803**			**32,803**
	$ 34,947	**$**	**$**	**$ 34,947**

	December 31, 2012	**Level 1**	**Level 2**	**Level 3**
Assets				
Cash equivalents	$ 15,580	$	$ 15,580	$
Marketable securities	18,580	$	18,580	
Restricted cash equivalents and marketable securities	374		374	
	$ 34,534	$	$ 34,534	$
Liability				
Share-based compensation liability				
Warrant liability				
	$	$	$	$

The following table presents a rollforward of the fair value of the share-based compensation liability which includes Level 3 measurements (in thousands):

	Three months ended September 30,		**Nine months ended September 30,**	
	2013	**2012**	**2013**	**2012**
Share-based compensation liability:				
Balance at beginning of period:	$	$	$	$
Change in fair value reclassified from equity	**1,273**		**1,273**	
Change in fair value included in share-based compensation expense	**871**		**871**	
Balance at end of period:	$ **2,144**	$	$ **2,144**	$

The fair value of the share-based compensation liability was calculated by grant award utilizing the Black-Scholes option-pricing model, using the following assumptions:

	July 26, 2013 through September 30, 2013
Risk-free interest rate	**1.40% - 2.16%**

Volatility	**115.7% - 123.4%**
Dividend yield	**nil**
Expected life in years	**2.8 - 6.5**

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The following table presents a rollforward of the fair value of the warrant liability which includes Level 3 measurements (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Warrant liability:				
Balance at beginning of period:	$ **12,208**	$	$ **16,195**	$
Change in fair value of warrant liability included in other (expense)/income	**20,595**		**16,608**	
Balance at end of period:	$ **32,803**	$	$ **32,803**	$

The fair value of the warrant liability was calculated utilizing the Black-Scholes option-pricing model, using the following assumptions:

	January 1, 2013		September 30, 2013	
	2011 Warrants	2012 Warrants	2011 Warrants	2012 Warrants
Risk-free interest rate	1.2%	1.4%	**1.4%**	**1.63%**
Volatility	107.5%	116.3%	**116.5%**	**115.4%**
Dividend yield	nil	nil	**nil**	**nil**
Expected life in years	3.3	4.9	**2.5**	**4.1**

Other financial assets

The Company s other financial assets consist of interest receivable, other receivables and security deposits. The carrying amount of these financial assets is a reasonable approximation of their fair value due to the short-term nature of these financial assets.

Other financial liabilities

The Company s other financial liabilities include accounts payable and accrued liabilities. The carrying value of the accounts payable and accrued liabilities approximates their fair value due to the short-term nature of these financial liabilities.

13. CONCENTRATION OF CREDIT RISK

The maximum exposure to credit risk of the Company at September 30, 2013 is the carrying value of its cash and cash equivalents, marketable securities, restricted cash equivalents and marketable securities, interest receivable, other receivables and security deposits. The Company has an investment policy that monitors the safety and preservation of investments made, which requires them to be highly rated and which limits the amount invested in any one issuer. The investments are reviewed regularly by the Audit Committee.

The Company also manages credit risk by maintaining bank accounts with reputable banks and financial institutions and investing only in investments from banking, governmental or highly rated companies with securities that are traded on active markets and are capable of immediate liquidation, subject to some minor market price variations upon sale.

Cash and cash equivalents and restricted cash were comprised of bankers acceptances, treasury bills, promissory notes, commercial papers, and term deposits at September 30, 2013 and at December 31, 2012. Cash and cash equivalents and restricted cash as of September 30, 2013 and December 31, 2012 were held in two Canadian chartered banks and one United States bank as follows (in thousands):

	September 30, 2013	December 31, 2012
Cash and cash equivalents	$ **7,616**	$ 18,403
Restricted cash	**371**	374
	$ **7,987**	$ 18,777

Management has determined that other receivables are collectible and has not recorded a provision against these amounts.

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The Company continues to have ongoing license and collaboration agreements with three partners. As per the terms of these agreements there were no revenues or expenses with these partners for the three or nine month periods ended September 30, 2013 and 2012.

14. SUBSEQUENT EVENTS

Restructuring

On October 1, 2013, the Company announced that it expects to terminate approximately 27 employees, or approximately 75% of the Company s total workforce, in connection with the closure of its Montreal, Quebec and Princeton, New Jersey offices (the Restructuring). The offices are being closed due to the Company consolidating its operations to the San Diego facility. The Company plans to partially offset this reduction in force by hiring additional personnel in the San Diego facility and by engaging third party services providers to perform certain functions. The Company expects the terminations and office closures to be substantially completed by March 31, 2014.

The Company expects that it will incur pre-tax charges of approximately $1.1 million relating to the office closures, consisting of approximately $0.9 million in one-time cash severance payments and related benefits, approximately $0.1 million in office closing costs, and approximately $0.1 million in asset impairment charges. The Company expects to recognize substantially all of the pre-tax charges by the first quarter of 2014. Approximately $1.1 million of these charges are expected to result in future cash expenditures.

Common Stock Offering

On October 29, 2013, the Company completed a public offering of common stock, or the Offering, for net proceeds of $53.0 million, after deducting underwriting discounts and commissions of $3.4 million and other estimated offering expenses of $0.5 million payable by the Company. The Company sold a total of 3,250,000 shares of its common stock in the Offering at a purchase price to the public of $17.50 per share. The underwriters were granted an option, exercisable for 30 days, to purchase up to 487,500 additional shares of common stock.

For its financial statements as of September 30, 2013 and for the nine-months then ended, the Company evaluated subsequent events through November 12, 2013, the date on which those financial statements were available to be issued.

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

Except for the historical information herein, the following discussion and analysis in this quarterly report on Form 10-Q contains forward-looking statements that involve risks and uncertainties. These statements include projections about our accounting and finances, plans and objectives for the future, future operating and economic performance and other statements regarding future events or conditions. These statements are not guarantees of future performance or events. Our actual results may differ materially from those discussed here. Factors that

could cause or contribute to differences in our actual results include those discussed under the caption Risk Factors, as well as those discussed elsewhere in this quarterly report on Form 10-Q or in our other public disclosures. You should consider carefully those cautionary factors, together with all of the other information included in this quarterly report on Form 10-Q. Each of the cautionary factors, either alone or taken together, could adversely affect our business, operating results and financial condition, as well as adversely affect the value of an investment in our common stock. There may be additional risks that we are not presently aware of or that we currently believe are immaterial which could also impair our business and financial position. We disclaim any obligation to update any forward-looking statements except as required by law.

We were incorporated under the laws of the State of Delaware on April 29, 2013. Our common shares are listed on The NASDAQ Capital Market since July 15, 2013 under the ticker symbol MRTX . On May 8, 2013, our Board of Directors approved and we entered into an arrangement agreement with MethylGene Inc. (MethylGene). Pursuant to the terms and conditions of the arrangement agreement which was consummated on June 28, 2013, the shareholders of MethylGene received one share of our common stock for every 50 common shares of MethylGene , which had the effect of a 50-for-1 reverse split of the common shares pursuant to a court-approved plan of arrangement under Section 192 of the *Canada Business Corporations Act*. Such transaction is referred to herein as the Arrangement. In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate adjustment was made to the exercise price. Upon consummation of the Arrangement on June 28, 2013, MethylGene became our wholly-owned subsidiary. As a result, the discussion contained in this Management s Discussion and Analysis of Financial Condition and Results of Operations reflect the consolidated operations of MethylGene.

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We are a holding company whose only asset is the stock of MethylGene . We conduct virtually all of our business operations through MethylGene and its wholly owned subsidiary, MethylGene US Inc.

Our historical functional currency was Canadian dollars as of December 31, 2012. Effective January 1, 2013, our functional currency became U.S. dollars. Our reporting currency is U.S. dollars and prior to January 1, 2013, for presentation purposes, assets and liabilities have been translated to U.S. dollars at exchange rates at the reporting date. Income and expenses have been translated to U.S. dollars at the average exchange rate for the period in which the transactions occurred. Equity transactions have been translated at the spot exchange rates on the date the transactions occurred. Exchange rate differences are recognized in a separate component of stockholders equity titled accumulated other comprehensive income.

Overview

Company Overview

We are a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific subsets of cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, multi-targeted kinase inhibitors with distinct target profiles that are in development to treat patients with non-small cell lung cancer, or NSCLC, and other solid tumors. MGCD265 is in Phase 1/2 clinical development and MGCD516 is in advanced preclinical development, with Phase 1 clinical development anticipated to begin in the first half of 2014. Mocetinostat is an orally-bioavailable, spectrum-selective histone deacetylase, or HDAC, inhibitor for the first line treatment of patients with myelodysplastic syndromes, or MDS, and potentially other indications. We are planning to initiate a Phase 3 clinical trial of mocetinostat in the second half of 2014 after obtaining a special protocol assessment, or SPA, from the FDA and subject to available funding or a partnership.

We believe that an increased understanding of the genomic factors that drive tumor cell growth can lead to the development of cancer drugs with increased efficacy while reducing side effects. We are leveraging this knowledge to develop targeted cancer therapies to address unmet needs in selected cancer patient populations. Our novel kinase inhibitors target specific mutations present only in cancer cells, and mocetinostat acts through epigenetic mechanisms important in treating certain cancers. We plan to identify additional opportunities by leveraging our deep scientific understanding of molecular drug targets and mechanisms of resistance and potentially in-licensing promising, early-stage novel drug candidates.

The following table summarizes key information about our three product candidates:

PRODUCT CANDIDATE	INDICATION	TARGETS	COMMERCIAL RIGHTS	STAGE OF DEVELOPMENT AND ANTICIPATED MILESTONES
MGCD265	Solid Tumors	Met, Axl, VEGFRs	Mirati: Global	• Initiate expansion cohorts Q1 2014 • Initiate Phase 2 Q4 2014

MGCD516	Solid Tumors	RET, TRK, DDR, EphRs, Met, Axl, VEGFRs	Mirati: Global	• Planned IND submission and initiate Phase 1 1H 2014 • Initiate expansion cohorts Q4 2014
Mocetinostat	MDS	HDACs 1, 2, 3, 11	Taiho: Certain Asian Territories Mirati: All Other Territories	• Initiate dose confirmation trial Q4 2013 • Obtain SPA for Phase 3 1H 2014 • Initiate Phase 3 2H 2014

MGCD265 is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of Met, Axl and VEGFRs. MGCD265 is in development for the treatment of solid tumors, with an initial focus on NSCLC and squamous cell carcinoma of the head and neck, or HNSCC. We have conducted single agent and combination dose escalation trials in 252 patients, with acceptable tolerability and promising early signs of clinical efficacy in patients with advanced solid tumors who have failed standard therapies. Our preclinical studies, in a variety of in vivo tumor models, have suggested that MGCD265 has relatively low toxicity and appears more potent than some of the leading approved kinase inhibitors, including Nexavar, Sutent and Xalkori. We have developed new formulations of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single agent clinical activity. Assuming one or more of the new formulations achieve sufficient patient exposure in ongoing studies, we intend to select one of the new formulations for introduction into ongoing dose escalation trials with the goal of identifying the maximum tolerated dose, or MTD, by early 2014. Following identification of the MTD, we plan to initiate dose expansion cohorts in patients selected for certain biomarkers.

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MGCD516 is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of RET, TRK, DDR and EphRs, as well as Met, Axl and VEGFRs, in development for the treatment of solid tumors. We plan to focus on solid tumors expressing RET, TRK and DDR, initially in NSCLC, and we plan to evaluate other tumor types where the profile of MGCD516 would suggest activity. MGCD516 is in advanced preclinical development. We plan to file an investigational new drug application, or IND, with the U.S. Food and Drug Administration, or FDA, and initiate a Phase 1 clinical trial of this product candidate in the first half of 2014, and identify the MTD and initiate expansion cohorts in patients selected for certain biomarkers by the end of 2014.

Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor for which we plan to conduct a dose confirmation trial starting in the fourth quarter of 2013, with the goal of initiating a Phase 3 clinical trial in the second half of 2014. We have completed 13 clinical trials which enrolled 437 patients with a variety of hematologic malignancies and solid tumors. We intend to seek a Special Protocol Assessment, or SPA, from the FDA prior to the initiation of our planned Phase 3 trial. This trial will evaluate mocetinostat for the first line treatment of patients with MDS in combination with Vidaza, a hypomethylating agent, or HMA. We believe that mocetinostat has the potential to be the first HDAC inhibitor to market for this indication.

Recent Developments

On October 1, 2013, we announced that we expect to terminate approximately 27 employees, or approximately 75% of our total workforce, in connection with the closure of our Montreal, Quebec and Princeton, New Jersey offices. The offices are being closed due to the consolidation of our operations to our San Diego facility. We plan to partially offset this reduction in force by hiring additional personnel in the San Diego facility and by engaging third-party services providers to perform certain functions. We expect the terminations and office closures to be substantially completed by March 31, 2014. We estimate that we will incur pre-tax charges of approximately $1.1 million relating to the office closures, consisting of approximately $0.9 million in one-time cash severance payments and related benefits, approximately $0.1 million in office closing costs, and approximately $0.1 million in asset impairment charges. We expect to recognize substantially all of the pre-tax charges by the first quarter of 2014. Approximately $1.1 million of these charges are expected to result in future cash expenditures. The numbers set forth above are estimates and may change as a result of a number of factors, including the timing of the terminations and office closures.

On October 29, 2013, we completed a public offering, or the Offering, of common stock for net proceeds of $53.0 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We sold a total of 3,250,000 shares of common stock in the Offering at a purchase price to the public of $17.50 per share. The underwriters were granted an option, exercisable for 30 days, to purchase up to 487,500 additional shares of common stock. We expect that our current cash, cash equivalents and marketable securities, together with the net proceeds from the Offering, will sustain our operations through the end of 2015.

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Liquidity Overview

We have incurred losses in each year since our inception. Our net losses were $41.6 million for the nine months ended September 30, 2013, and $20.3 million and $9.8 million for the years ended December 31, 2012 and 2011, respectively. As of September 30, 2013 we had an accumulated deficit of $187.2 million. Substantially all of our operating losses resulted from expenses incurred in connection with our drug candidate development programs, our research activities and general and administrative costs associated with our operations.

To fund future operations we may need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot assure you that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

Critical Accounting Policies and Significant Judgments and Estimates

Our management s discussion and analysis of financial condition and results of operations are based upon our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make significant estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosures. On an ongoing basis, our actual results may differ significantly from our estimates.

There were no significant changes in critical accounting policies from those at December 31, 2012. The financial information as of September 30, 2013 should be read in conjunction with the financial statements for the year ended December 31, 2012, and the related notes thereto, contained in our Registration Statement on Form 10 (No. 001-35921), originally filed with the SEC on May 10, 2013, as amended.

For a further discussion of our critical accounting policies, see Item 2. Financial Information under the heading Management s Discussion and Analysis of Financial Condition and Results of Operations contained in our Registration Statement on Form 10 (No. 001-35921), originally filed with the SEC on May 10, 2013, as amended.

Results of Operations

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

The following table summarizes our results of operations for the three months ended September 30, 2013 and 2012 (in thousands):

	Three Months Ended September 30, 2013	2012	Increase (Decrease)
Research and development expenses, net	$ **5,492**	$ 4,249	$ 1,243
General and administrative expenses	**3,710**	1,717	1,993
Other (expense)/income, net	**(20,141)**	34	(20,175)

Research and Development Expenses

Our research and development efforts have been focused on oncology, including MGCD265, MGCD516 and mocetinostat. The following table summarizes our research and development expenses for the three months ended September 30, 2013 and 2012 (in thousands):

	Three months ended September 30, 2013	2012
Third-party clinical development costs:		
MGCD265	**$ 1,158**	1,911
MGCD290	**469**	796
Mocetinostat	**972**	7
MGCD516	**881**	
Total third-party clinical development costs:	**3,480**	2,714
Internal clinical development costs	**822**	632
Total clinical development	**4,302**	3,346
Non-clinical research and development	**1,386**	1,039
Research and development, gross	**5,688**	4,385
Less: Investment tax credits	**(196)**	(136)
Research and development, net	**5,492**	**$** 4,249

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Net research and development expenses were $5.5 million for the three months ended September 30, 2013 compared to $4.2 million for the same period in 2012. The increase primarily reflects increased costs associated with a dose confirmation clinical trial for mocetinostat planned to commence in the fourth quarter of 2013, and costs associated with preparation for an Investigational New Drug, or IND, application for MGCD516, planned for the first half of 2014. Partially offsetting these increases were reduced costs for MGCD265 due to decreased clinical drug product manufacturing activities during the third quarter of 2013 compared to the same period of 2012, reduced costs for MGCD290 which we are no longer actively pursuing internally and an increase in investment tax credits due to our higher level of investment in research and development activities.

General and Administrative Expenses

General and administrative expenses were $3.7 million for the three months ended September 30, 2013 compared to $1.7 million for the same period in 2012. The increase primarily reflects the increase of share-based compensation costs related to the reclassification of stock options denominated in the Canadian dollar under the 1997 Equity Plan that were granted to U.S.- based employees and directors effective July 26, 2013 (refer to Note 3 of the consolidated financial statements for more details), establishing a new management team in San Diego and costs associated with listing on The NASDAQ Capital Market, which was effective on July 15, 2013.

Other (expense) income, net

Other (expense) income, net was net expense of $20.1 million for the three months ended September 30, 2013 compared to net income of $34,000 for the same period in 2012. The increase primarily reflects a loss of $20.6 million from the change in fair value of our warrant liability(refer to Note 3 of the accompanying consolidated financial statements for more details), partially offset by a foreign exchange gain of $411,000 primarily due to the transition to the U.S. dollar as the functional currency and net financial income of $43,000.

Comparison of the Nine Months Ended September 30, 2013 and 2012

The following table summarizes the results of our operations for the nine months ended September 30, 2013 and 2012 (in thousands):

	Nine Months Ended September 30, 2013	2012	Increase
Research and development expenses, net	**$ 15,477**	$ 10,105	$ 5,372
General and administrative expenses	**8,616**	4,019	4,597
Other (expense) income, net	**(17,418)**	172	(17,590)

Research and Development Expenses

Our research and development efforts during the nine months ended September 30, 2013 were focused on oncology, including MGCD265, MGCD516 and mocetinostat . During the nine months ended September 30, 2012 we were focused on MGCD265 for oncology and MGCD290 for antifungal indications. The following table summarizes our research and development expenses for the nine months ended September 30, 2013 and 2012 (in thousands):

	Nine months ended September 30,	
	2013	**2012**
Third-party clinical development costs:		
MGCD265	**$ 5,075**	$ 4, 387
MGCD290	**1,467**	1,970
Mocetinostat	**1,587**	39
MGCD516	**1,339**	
Total third-party clinical development costs:	**9,468**	6,396
Internal clinical development costs	**2,802**	2,826
Total clinical development	**12,270**	9,222
Non-clinical research and development	**3,885**	2,413
Research and development, gross	**16,155**	11,635
Less: Investment tax credits	**(678)**	(1,530)
Research and development, net	**$ 15,477**	$ 10,105

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Net research and development expenses were $15.5 million for the nine months ended September 30, 2013 compared to $10.1 million for the same period in 2012. The increase primarily reflects the same factors that influenced similar fluctuations in the three months ended September 30, 2013 discussed above as well as costs associated with ongoing formulation development for MGCD265 and costs associated with management changes implemented in the first nine months of 2013. The investment tax credits for the nine months ended September 30, 2013 declined compared to the same period in 2012 because the prior year included a favorable adjustment associated with the completion of an audit by the provincial tax authority in Canada.

General and Administrative Expenses

General and administrative expenses were $8.6 million for the nine months ended September 30, 2013 compared to $4.0 million for the same period in 2012. The increase primarily reflects the same factors that influenced similar fluctuations in the three months ended September 30, 2013 as well as costs associated with the Arrangement.

Other (Expense) Income, Net

Other expense, net was $ 17.4 million for the nine months ended September 30, 2013 compared to other income, net of $172,000 for the same period in 2012. The increased net expense primarily reflects a loss of $16.6 million from the change in fair value of our warrant liability (refer to Note 3 of the accompanying consolidated financial statements for further details) and a foreign exchange loss of $0.9 million primarily due to the transition to the U.S. dollar as the functional currency.

Liquidity and Capital Resources

Since our inception, our operations have been primarily financed through public and private sales of our equity and payments received under our collaboration arrangements. We have devoted our resources to funding research and development programs, including discovery research, preclinical and clinical development activities.

We have incurred losses in each year since our inception. Our net losses were $41.6 million for the nine months ended September 30, 2013, and $20.3 million and $9.8 million for the years ended December 31, 2012 and 2011, respectively. As of September 30, 2013 we had an accumulated deficit of $187.2 million. Substantially all of our operating losses resulted from expenses incurred in connection with our drug candidate development programs, our research activities and general and administrative costs associated with our operations.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. In the near term, we anticipate that our expenses will increase as we:

- advance our two lead kinase programs, MGCD265 and MGCD516 in development for the treatment of solid tumors;

- advance mocetinostat, our later stage drug candidate in development for the treatment of hematologic malignancies;

- evaluate opportunities for the expansion of our oncology portfolio, including evaluating and possibly executing in-licensing and partnering transactions;

- continue our translational science research efforts;

- maintain, expand and protect our intellectual property portfolio; and

- provide general and administrative support for our operations.

At September 30, 2013 we had $15.4 million of cash and cash equivalents, marketable securities, and restricted cash and marketable securities compared to $37.4 million at December 31, 2012. On October 29, 2013, we completed a public offering of common stock, or the Offering, for net proceeds of $53.0 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We sold a total of 3,250,000 shares of our common stock in the Offering at a purchase price to the public of $17.50 per share. The underwriters were granted an option, exercisable for 30 days, to purchase up to 487,500 additional shares of common stock. We expect that our current cash, cash equivalents and marketable securities, together with the net proceeds from the Offering, will sustain our operations through the end of 2015.

To fund future operations we may need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot assure you that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

Cash Flows for the Nine Months Ended September 30, 2013 and 2012

Operating Activities

Cash used for operating activities for the nine months ended September 30, 2013 was $21.7 million compared to $11.5 million for the same period in 2012. The increase relates primarily to the increased operating costs in the first nine months of 2013, including non-recurring costs associated with the previously discussed Arrangement, the NASDAQ listing and ongoing management changes, compared to the same period in 2012.

Investing Activities

Investing activities provided cash of $10.9 million and $6.9 million for the nine months ended September 30, 2013 and 2012, respectively. Investing activities consist primarily of purchases, sales and maturities of marketable securities and purchases of property and equipment. The increase in cash flows provided by investing activities is largely due to an increase in maturities of marketable securities, offset by an increase in purchases of marketable securities.

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Financing Activities

Financing activities provided cash of $22,000 and used cash of $16,000 for the nine months ended September 30, 2013 and 2012, respectively. Financing activities consist primarily of net proceeds from the exercise of warrants and costs for reorganization.

Off-Balance Sheet Arrangements

During the nine months ended September 30, 2013, we did not have any off-balance sheet arrangements (as defined by applicable SEC regulations) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

ITEM 4. Controls and Procedures

As of September 30, 2013, an evaluation was performed under the supervision and with the participation of our management, including our President and Chief Executive Officer (referred to as our CEO) and our Executive Vice President, and Chief Operations Officer (referred to as our COO), of the effectiveness of the design and operation of our disclosure controls and procedures. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management necessarily is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on that evaluation, our management, including our CEO and COO, concluded that our disclosure controls and procedures were effective at a reasonable level of assurance as of September 30, 2013.

Our management does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, or misstatements due to error, if any, within the company have been detected. While we believe that our disclosure controls and procedures and internal control over financial reporting are and have been effective, in light of the foregoing we intend to continue to examine and refine our disclosure controls and procedures and internal control over financial reporting.

An evaluation was also performed under the supervision and with the participation of our management, including our CEO and COO, 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 and that has materially affected, or is reasonably likely to affect, our internal control over financial reporting.

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 quarterly report on Form 10-Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Relating to Our Financial Position and Capital Requirements

We will require additional financing and may be unable to raise sufficient capital, which could lead us to delay, reduce or abandon development programs or commercialization.

Our operations have consumed substantial amounts of cash since inception. Our net research and development expenses were $15.5 million for the nine months ended September 30, 2013, and $15.1 million and $8.9 million for the years ended December 31, 2012 and 2011, respectively. We believe that our cash and cash equivalents and marketable securities as of September 30, 2013, together with the $53.0 million of net proceeds from the public offering of common stock that we completed on October 29, 2013, will sustain our operations through the end of 2015. Pursuant to our current plans, we do not anticipate initiating Phase 3 trials with mocetinostat absent additional financing or the establishment of a collaboration for late-stage development. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. We will require substantial additional capital to pursue additional clinical development for our lead clinical programs, including conducting clinical trials, manufacturing clinical supplies and potentially developing other assets in our pipeline, and, if we are successful, to commercialize any of our current product candidates. If the U.S. Food and Drug Administration, or FDA, or any foreign regulatory agency, such as the European Medicines Agency, or EMA, requires that we perform studies or trials in addition to those that we currently anticipate with respect to the development of our product candidates, or repeat studies or trials, our expenses would further increase beyond

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what we currently expect. Any delay resulting from such further or repeat studies or trials could also result in the need for additional financing. We may not be able to adequately finance our development programs, which could limit our ability to move our programs forward in a timely and satisfactory manner or require us to abandon the programs, any of which would harm our business, financial condition and results of operations. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds we will require to complete research and development and commercialize our product candidates.

If we are unable to obtain funding from equity offerings or debt financings, including on a timely basis, we may be required to (1) seek collaborators for one or more of our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available; (2) relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves; or (3) significantly curtail one or more of our research or development programs or cease operations altogether.

We are a clinical stage company with no approved products and no historical product revenues. Consequently, we expect that our financial and operating results will vary significantly from period to period.

We are a clinical-stage development company that has incurred losses since its inception and expect to continue to incur substantial losses in the foreseeable future. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty.

Our actual financial condition and operating results have varied significantly in the past and are expected to continue to fluctuate significantly from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations include:

- the success of our clinical trials through all phases of clinical development;
- delays in the commencement, enrollment and timing of clinical trials;
- our ability to secure and maintain collaborations, licensing or other arrangements for the future development and/or commercialization of our product candidates, as well as the terms of those arrangements;
- our ability to obtain, as well as the timeliness of obtaining, additional funding to develop our product candidates;
- the results of clinical trials or marketing applications for product candidates that may compete with our product candidates;

- competition from existing products or new products that may receive marketing approval;

- potential side effects of our product candidates that could delay or prevent approval or cause an approved drug to be taken off the market;

- any delays in regulatory review and approval of our product candidates;

- our ability to identify and develop additional product candidates;

- the ability of patients or healthcare providers to obtain coverage or sufficient reimbursement for our products;

- our ability, and the ability of third parties such as Clinical Research Organizations, or CROs, to adhere to clinical study and other regulatory requirements;

- the ability of third-party manufacturers to manufacture our product candidates and key ingredients needed to conduct clinical trials and, if approved, successfully commercialize our products;

- the costs to us, and our ability as well as the ability of any third-party collaborators, to obtain, maintain and protect our intellectual property rights;

- costs related to and outcomes of potential intellectual property litigation;

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- our ability to adequately support future growth;

- our ability to attract and retain key personnel to manage our business effectively; and

- our ability to build our finance infrastructure and improve our accounting systems and controls.

Accordingly, the likelihood of our success must be evaluated in light of many potential challenges and variables associated with a clinical-stage company, many of which are outside of our control, and past operating or financial results should not be relied on as an indication of future results. Fluctuations in our operating and financial results could cause our share price to decline. It is possible that in some future periods, our operating results will be above or below the expectations of securities analysts or investors, which could also cause our share price to decline.

We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have never generated any revenue from product sales and may never be profitable.

We have derived limited revenue from our research and licensing agreements which have not been sufficient to cover the substantial expenses we have incurred in our efforts to develop our product candidates. Consequently, we have accumulated net losses since inception in 1995. Our net loss for the nine months ended September 30, 2013 was $41.6 million and for the twelve months ended December 31, 2012 and 2011 it was $20.3 million and $9.8 million, respectively. As of September 30, 2013, we had an accumulated deficit of $187.2 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders equity and working capital. Such losses are expected to increase in the future as we continue the development of our product candidates and seek regulatory approval and commercialization for our product candidates. We are unable to predict the extent of any future losses or when we will become profitable, if ever. Even if we do achieve profitability, we may not be able to sustain or increase profitability on an ongoing basis.

We do not anticipate generating revenue from sales of products for the foreseeable future, if ever. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. If one or more of our product candidates is approved for commercial sale and we retain commercial rights, we anticipate incurring significant costs associated with commercializing any such approved product candidate. Therefore, even if we are able to generate revenue from the sale of any approved product, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing development and clinical trial programs for our product candidates;

- entering into collaboration and license agreements;

- seeking and obtaining marketing approvals for any product candidates that successfully complete clinical trials;

- establishing and maintaining supply and manufacturing relationships with third parties;

- successfully commercializing any product candidates for which marketing approval is obtained; and

- successfully establishing a sales force, marketing and distribution infrastructure.

Raising additional funds through debt or equity financing will be dilutive and raising funds through licensing agreements may be dilutive, restrict operations or relinquish proprietary rights.

To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Existing stockholders may not agree with our financing plans or the terms of such financings. Moreover, the incurrence of debt financing could result in a substantial portion of our operating cash flow being dedicated to the payment of principal and interest on such indebtedness and could impose restrictions on our operations. In addition, if we raise additional funds through collaboration and licensing arrangements, it may be necessary to relinquish potentially valuable rights to our products or proprietary technologies, or to

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grant licenses on terms that are not favorable to us. Additional funding may not be available to us on acceptable terms, or at all.

We may incur losses associated with foreign currency fluctuation.

Our headquarters were previously located in Canada and many of our material contracts were entered into in Canada. A significant portion of our expenditures are in foreign currencies, most notably in Canadian dollars; therefore, we are subject to foreign currency fluctuations which may, from time to time, impact (positively or negatively) our financial position and results of operations. Exchange rates can fluctuate significantly and cannot be easily predicted; thus, we may experience significant shifts in currency exchange variances in the future. We maintain bank accounts in both Canadian dollars and U.S. dollars and do not hedge our positions. Our functional currency at December 31, 2012 was the Canadian dollar and based on extensive analysis of projected expenses we changed our functional currency to the U.S. dollar effective January 1, 2013.

As a public company in the United States, we are subject to the Sarbanes-Oxley Act. We can provide no assurance that we will, at all times, in the future be able to report that our internal controls over financial reporting are effective.

Companies that file reports with the Securities and Exchange Commission, or the SEC, including us, are subject to the requirements of Section 404 of the Sarbanes-Oxley Act of 2002. Section 404 requires management to establish and maintain a system of internal control over financial reporting, and annual reports on Form 10-K filed under the Securities Exchange Act of 1934, as amended, or the Exchange Act, must contain a report from management assessing the effectiveness of a company s internal control over financial reporting.

As a smaller reporting company (as defined in the Exchange Act) we will be required to comply with Section 404 of the Sarbanes-Oxley Act although, as an emerging growth company (as defined in the JOBS Act) and a smaller reporting company, we are not required to comply with Section 404(b) which requires attestation from our external auditors on our internal control over financial reporting. We are subject to Section 404(a), which requires management to provide a report regarding the effectiveness of internal controls. We were previously listed on the Toronto Stock Exchange, or TSX, from June 2004 until July 2013 and were subject to similar governance requirements under Multi-lateral Instrument 52-109. We are required to review all of our control processes to align them to the SOX 404 requirements. Failure to provide assurance that our financial controls are effective could lead to lack of confidence by investors which could lead to a lower share price. When and if we become a large accelerated filer or an accelerated filer and are no longer an emerging growth company (each as defined in the Exchange Act or the Securities Act of 1933, as amended, or the Securities Act), our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we may need to upgrade our systems including information technology, implement additional financial and management controls, reporting systems and procedures, and hire additional accounting and finance staff.

We will incur significant increased costs as a result of operating as a U.S. public company and continuing to be a Canadian reporting issuer.

Although we de-listed from the TSX effective as of July 26, 2013, we will continue to be subject to Canadian reporting obligations. Our Canadian reporting obligations will continue until we meet certain prescribed thresholds which would allow us to apply to cease being a

Canadian reporting issuer. We may incur significant additional accounting, reporting and other expenses in order to maintain our listing on The NASDAQ Capital Market, and fulfill our obligations as a Canadian reporting issuer. For example, we may incur additional expenses if we are required to continue to present our financial information according to International Financial Reporting Standards in Canada, as well as according to U.S. generally accepted accounting principles. In addition, as a U.S. listed public company, we will incur significant additional legal, accounting and other expenses that we did not incur as a company listed on the TSX. Shareholder 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. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, any new regulations or disclosure obligations may increase our legal and financial compliance costs and will make some activities more time-consuming and costly. Incremental recurring external costs associated with being a publicly traded company in the United States are estimated to be approximately $0.5 million per year, consisting primarily of increased legal, accounting and insurance costs.

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

We are an emerging growth company. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

For as long as we continue to be an emerging growth company, we intend to take advantage of certain other exemptions from various reporting requirements that are applicable to other public companies including, but not limited to, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory stockholder vote on executive compensation and any golden parachute payments not previously approved, exemption from the requirement of auditor attestation in the assessment of our internal control over financial reporting and exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board. If we do, the information that we provide stockholders may be different than what is available with respect to other public companies. We cannot predict if investors will find our common stock less attractive because we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less-active trading market for our common stock and our stock price may be more volatile.

We will remain an emerging growth company until the earliest of (1) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds $700 million as of the end of the second fiscal quarter, (2) the end of the fiscal year in which we have total annual gross revenue of $1 billion or more during such fiscal year, (3) the date on which we issue more than $1 billion in non-convertible debt in a three-year period or (4) the end of the fiscal year following the fifth anniversary of the date of the first sale of our common stock pursuant to an effective registration statement filed under the Securities Act.

Decreased disclosures in our SEC filings due to our status as an emerging growth company may make it harder for investors to analyze our results of operations and financial prospects.

We are a smaller reporting company and we cannot be certain if the reduced disclosure requirements applicable to smaller reporting companies will make our common stock less attractive to investors.

We are currently a smaller reporting company, meaning that we are not an investment company, an asset-backed issuer, or a majority-owned subsidiary of a parent company that is not a smaller reporting company and had a public float of less than $75 million and annual revenue of less than $50 million during the most recently completed fiscal year. In the event that we are still considered a smaller reporting company at such time as we cease being an emerging growth company, we will be required to provide additional disclosure in our SEC filings. However, similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosures in their filings, are exempt from the provisions of Section 404(b) of the Sarbanes-Oxley Act requiring that independent registered public accounting firms provide an attestation report on the effectiveness of internal control over financial reporting, and have certain other decreased disclosure obligations in their SEC filings, including, among other things, only being required to provide two years of audited financial statements in annual reports.

Decreased disclosures in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects.

Risks Relating to Our Business and Industry

Our research and development programs and product candidates are at an early stage of development. As a result we are unable to predict if or when we will successfully develop or commercialize our product candidates.

Our clinical-stage product candidates as well as our other pipeline assets are at an early stage of development and will require significant further investment and regulatory approvals prior to commercialization. We currently have no product candidates beyond Phase 2 clinical trials. MGCD265 is currently in Phase 1 and Phase 1/2 clinical trials, and MGCD516 is in advanced preclinical development. Each of our product candidates will require additional clinical development, management of clinical, preclinical and manufacturing activities, obtaining regulatory approval, obtaining manufacturing supply, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenues from product sales. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for

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any of our product candidates. In addition, some of our product development programs contemplate the development of companion diagnostics. Companion diagnostics are subject to regulation as medical devices and we may be required to obtain marketing approval for accompanying companion diagnostics before we may commercialize our product candidates. We plan on conducting a dose confirmation trial and obtaining an SPA with the FDA prior to initiating Phase 3 trials with mocetinostat. In addition, we do not anticipate initiating a Phase 3 clinical trial with mocetinostat absent additional financing or the establishment of a collaboration for late-stage development.

Even if we obtain the required financing or establish a collaboration to enable us to conduct late-stage clinical development of our product candidates and pipeline assets, we cannot be certain that such clinical development would be successful, or that we will obtain regulatory approval or be able to successfully commercialize any of our product candidates and generate revenue. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and the clinical trial process may fail to demonstrate that our product candidates are safe and effective for their proposed uses. Any such failure could cause us to abandon further development of any one or more of our product candidates and may delay development of other product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Any delay in, or termination of, our clinical trials will delay and possibly preclude the filing of any new drug applications, or NDAs, with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenue.

We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon our or our future collaborators ability to obtain regulatory approval of the companion diagnostics to be used with our product candidates, if required, 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 patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

All of our product candidates are subject to extensive regulation, which can be costly and time consuming, cause delays or prevent approval of such product candidates for commercialization.

The clinical development of product candidates is subject to extensive regulation by the FDA in the United States and by comparable regulatory authorities in foreign markets. Product development is a very lengthy and expensive process, and its outcome is inherently uncertain. The product development timeline can vary significantly based upon the product candidate s novelty and complexity. Regulations are subject to change and regulatory agencies have significant discretion in the approval process.

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States, Canada and other countries where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of protocols and human testing, the approval of manufacturing facilities, safety of the product candidates, testing procedures and controlled research, review and approval of manufacturing, preclinical and clinical data prior to marketing approval including adherence to good manufacturing practices, or GMP, during production and storage as well as regulation of marketing activities including advertising and labeling.

In order to obtain regulatory approval for the commercial sale of any of our product candidates, we must demonstrate through preclinical studies and clinical trials that the potential product is safe and effective for use in humans for each target indication. The failure to adequately demonstrate the safety and efficacy of a product under development could delay or prevent regulatory approval of our product candidates.

No assurance can be given that current regulations relating to regulatory approval will not change or become more stringent in the United States or foreign markets. Regulatory agencies may also require that additional trials be run in order to provide additional information regarding the safety or efficacy of any compound for which we seek regulatory approval. Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Regulatory agencies could become more risk adverse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

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We may not be successful in establishing development and commercialization collaborations which could adversely affect, and potentially prohibit, our ability to develop our product candidates.

Because developing pharmaceutical products, conducting clinical trials, obtaining regulatory approval, establishing manufacturing capabilities and marketing approved products are expensive, we may seek to enter into collaborations with companies that have more resources and experience in order to continue to develop and commercialize our product candidates. We also may be required due to financial or scientific constraints to enter into additional corporate collaboration agreements to research and/or to develop and commercialize our product candidates. The establishment and realization of such collaborations may be not be possible or may be problematic. There can be no assurance that we will be able to establish such additional collaborations on favorable terms, if at all, or that our current or future collaborative arrangements will be successful or maintained for any specific product candidate or indication. If we are unable to reach successful agreements with suitable partners for the ongoing development and commercialization of our product candidates, we may face increased costs, we may be forced to limit the scope and number of our product candidates we can commercially develop or the territories in which we commercialize such product candidates and we may be unable to commercialize products or programs for which a suitable partner cannot be found. If we fail to achieve successful partnerships, our operating results and financial condition will be materially and adversely affected.

In addition, the terms of any collaboration agreements may place restrictions on our activities with respect to other products, including by limiting our ability to grant licenses or develop products with other third parties, or in different indications, diseases or geographical locations, or may place additional obligations on us with respect to development or commercialization of our product candidates. If we fail to comply with or breach any provision of a collaborative or license agreement, a collaborator may have the right to terminate, in whole or in part, such agreement or to seek damages.

Some of our collaboration agreements are complex and involve sharing or division of ownership of certain data, know-how and intellectual property rights among the various parties. Accordingly our collaborators could interpret certain provisions differently than we or our other partners which could lead to unexpected or inadvertent disputes with partners. In addition, these agreements might make additional partnering or mergers and acquisitions difficult.

There is no assurance that a collaborator who is acquired by a third party would not attempt to change certain contract provisions that could negatively affect our collaboration. The acquiring company may also not accept the terms or assignment of our contracts and may seek to terminate the agreements. Any one of our partners could breach covenants, restrictions and/or sub-license agreement provisions leading us into disputes and potential breaches of our agreements with other partners.

If we or third parties are unable to successfully develop companion diagnostics for our kinase inhibitor product candidates, or experience significant delays in doing so, we may not achieve marketing approval or realize the full commercial potential of such product candidates.

A key part of our strategy for our kinase inhibitor development program, including MGCD265 and MGCD516, is to identify patients or types of tumors that express specific genetic markers, which will require the use and development of companion diagnostics. We expect that the FDA and comparable foreign regulatory authorities will require the regulatory approval of a companion diagnostic as a condition to approving these product candidates. We do not have experience or capabilities in developing or commercializing diagnostics and plan to rely in large part on third parties to perform these functions. We do not currently have any long-term arrangements in place with any third party to develop or commercialize companion diagnostics for any of our therapeutic product candidates.

Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as medical devices and will likely require separate regulatory approval prior to commercialization.

If we or third parties are unable to successfully develop companion diagnostics for our kinase inhibitor product candidates, or experience delays in doing so:

- the development of these product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;

- these product candidates may not receive marketing approval if their safe and effective use depends on a companion diagnostic; and

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- we may not realize the full commercial potential of these product candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients or types of tumors with the specific genetic alterations targeted by these product candidates.

Even if our kinase inhibitor product candidates and any associated companion diagnostics are approved for marketing, the need for companion diagnostics may slow or limit adoption of our product candidates. Although we believe genetic testing is becoming more prevalent in the diagnosis and treatment of cancer, our product candidates may be perceived negatively compared to alternative treatments that do not require the use of companion diagnostics, either due to the additional cost of the companion diagnostic or the need to complete additional procedures to identify genetic markers prior to administering our product candidates.

If any of these events were to occur, our business and growth prospects would be harmed, possibly materially.

We may not be able to obtain an SPA prior to initiating Phase 3 clinical trials of mocetinostat. Even if obtained, an SPA would not guarantee any particular outcome from regulatory review.

We plan to submit an SPA to the FDA for the planned Phase 3 development of mocetinostat. The FDA s SPA process creates a written agreement between the sponsoring company and the FDA regarding clinical trial design and other clinical trial issues that can be used to support approval of a product candidate. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, SPA agreements are not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, SPAs are not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. We cannot guarantee that we will obtain an SPA for the Phase 3 development of mocetinostat or that an SPA, if obtained, would ultimately aid in obtaining regulatory approval.

We rely upon third-party contractors and service providers for the execution of some aspects of our development programs. Failure of these collaborators to provide services of a suitable quality and within acceptable timeframes may cause the delay or failure of our development programs.

We outsource certain functions, tests and services to CROs, medical institutions and collaborators as well as outsourcing manufacturing to collaborators and/or contract manufacturers and we rely on third parties for quality assurance, clinical monitoring, clinical data management and regulatory expertise. In particular, we rely on CROs to run our clinical trials on our behalf. There is no assurance that such individuals or organizations will be able to provide the functions, tests, drug supply or services as agreed upon or to acceptable quality standards, and we could suffer significant delays in the development of our products or processes.

In some cases there may be only one or few providers of such services, including clinical data management or manufacturing services. In addition, the cost of such services could increase significantly over time. We rely on third parties as mentioned above to enroll qualified patients and conduct, supervise and monitor our clinical trials. Our reliance on these third parties and collaborators for clinical development activities reduces our control over these activities, but does not relieve us of our regulatory responsibilities, including ensuring that our clinical trials are

conducted in accordance with good clinical practices, or GCP, regulations and the investigational plan and protocols contained in the regulatory agency applications. In addition, these third parties may not complete activities on schedule or may not manufacture compounds under GMP conditions. Preclinical studies may not be performed or completed in accordance with good laboratory practices, or GLP, regulatory requirements or our trial design. If we or our CROs fail to comply with GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving any marketing applications. If these third parties or collaborators do not successfully carry out their contractual duties or meet expected deadlines, obtaining regulatory approval for manufacturing and commercialization of our product candidates may be delayed or prevented. We rely substantially on third-party data managers for our clinical trial data. There is no assurance that these third parties will not make errors in the design, management or retention of our data or data systems. There is no assurance these third parties will pass FDA or regulatory audits, which could delay or prohibit regulatory approval.

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Our CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could harm our competitive position. If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. Further, switching or adding additional CROs involves additional cost and requires management time and attention. In addition, there is a natural transition period when a new CRO commences work. As a result, delays may occur, which could 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 challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

The timelines of our clinical trials may be impacted by numerous factors and any delays may adversely affect our ability to execute our current business strategy.

Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. We may experience delays in clinical trials at any stage of development and testing of our product candidates. Our planned clinical trials may not begin on time, have an effective design, enroll a sufficient number of subjects, or be completed on schedule, if at all.

Events which may result in a delay or unsuccessful completion of clinical trials include:

- inability to raise funding necessary to initiate or continue a trial;
- delays in obtaining regulatory approval to commence a trial;
- delays in reaching agreement with the FDA on final trial design;
- imposition of a clinical hold following an inspection of our clinical trial operations or trial sites by the FDA or other regulatory authorities;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in obtaining required institutional review board approval at each site;
- delays in recruiting suitable patients to participate in a trial;
- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- delays caused by subjects dropping out of a trial due to side effects or otherwise;
- clinical sites dropping out of a trial to the detriment of enrollment;
- time required to add new clinical sites; and
- delays by our contract manufacturers to produce and deliver a sufficient supply of clinical trial materials.

For example, due to the targeted indications and patient population we intend to focus on for development of our kinase inhibitor product candidates, the number of study sites and patient populations available to us may be relatively limited, and therefore enrollment of suitable patients to participate in clinical trials for these product candidates may take longer than would be the case if we were pursuing broader indications or patient populations.

If initiation or completion of any of our clinical trials for our product candidates are delayed for any of the above reasons, our development costs may increase, our approval process could be delayed, any periods after commercial launch and before expiration of patent protection may be reduced and our competitors may have more time to bring products to market before we do. Any of these events could impair the commercial potential of our product candidates and could have a material adverse effect on our business.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, 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 product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Results of our trials could reveal a high and unacceptable severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial, or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, 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;

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- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of any product candidate, if approved, and could significantly harm our business, results of operations and prospects.

We are and continue to be subject to stringent government regulations concerning the clinical testing of our products. We will also continue to be subject to government regulation of any product that receives regulatory approval.

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States and other countries where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of protocols and human testing, the approval of manufacturing facilities, testing procedures and controlled research, the review and approval of manufacturing, preclinical and clinical data prior to marketing approval, including adherence to GMP during production and storage, and marketing activities including advertising and labeling.

Clinical trials may be delayed or suspended at any time by us or by the FDA or other similar regulatory authorities if it is determined at any time that patients may be or are being exposed to unacceptable health risks, including the risk of death, or if compounds are not manufactured under acceptable GMP conditions or with acceptable quality. Current regulations relating to regulatory approval may change or become more stringent. The agencies may also require additional trials be run in order to provide additional information regarding the safety, efficacy or equivalency of any compound for which we seek regulatory approval.

Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with GMPs and GCPs for any clinical trials that we conduct post-approval. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Similar restrictions are imposed in foreign markets. Regulatory agencies could become more risk adverse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved.

If we, or any future marketing collaborators or contract manufacturers, fail to comply with applicable regulatory requirements, we may be subject to sanctions including fines, product recalls or seizures and related publicity requirements, injunctions, total or partial suspension of production, civil penalties, suspension or withdrawals of previously granted regulatory approvals, warning or untitled letters, refusal to approve pending applications for marketing approval of new products or of supplements to approved applications, import or export bans or restrictions, and criminal prosecution and penalties. Any of these penalties could delay or prevent the promotion, marketing or sale of our products and product candidates.

The FDA s policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. 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, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We have no experience in commercial manufacturing and depend on others for the production of our product candidates at suitable levels of quality and quantity. Any problems or delays in the manufacture of our products would have a negative impact on our ability to successfully execute our development and commercialization strategies.

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 product candidates on a clinical or commercial scale. We rely on collaborators and/or third parties for development, scale-up, formulation, optimization, management of clinical trial and commercial scale manufacturing and

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commercialization. There are no assurances we can scale-up, formulate or manufacture any product candidate in sufficient quantities with acceptable specifications for the conduct of our clinical trials or for the regulatory agencies to grant approval of such product candidate. We have not yet commercialized any products and have no commercial manufacturing experience. To be successful, our products must be properly formulated, scalable, stable and safely manufactured in clinical trial and commercial quantities in compliance with GMP and other regulatory requirements and at acceptable costs. Should any of our suppliers or our collaborators be unable to supply or be delayed in supplying us with sufficient supplies, no assurance can be given that we will be able to find alternative means of supply in a short period of time. Should such parties operations suffer a material adverse effect, the manufacturing of our products would also be adversely affected. Furthermore, key raw materials could become scarce or unavailable. There may be a limited number of third parties who can manufacture our products. We may not be able to meet specifications previously established for compounds during scale-up and manufacturing.

Our reliance on third parties to manufacture our product candidates will expose us and our partners to risks including the following, any of which could delay or prevent the commercialization of our products, result in higher costs, or deprive us of potential product revenue:

- Contract manufacturers can encounter difficulties in achieving the scale-up, optimization, formulation, volume production of a compound as well as maintaining quality control with appropriate quality assurance. They may also experience shortages of qualified personnel. Contract manufacturers are required to undergo a satisfactory GMP inspection prior to regulatory approval and are obliged to operate in accordance with FDA, International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, European and other nationally mandated GMP regulations and/or guidelines governing manufacturing processes, stability testing, record keeping and quality standards. A failure of these contract manufacturers to follow GMP and to document their adherence to such practices or failure of an inspection by a regulatory agency may lead to significant delays in the availability of material for clinical study, leading to delays in our trials.

- For each of our current product candidates we will initially rely on a limited number of contract manufacturers. Changing these or identifying future manufacturers may be difficult. Changing manufacturers requires re-validation of the manufacturing processes and procedures in accordance with FDA, ICH, European and other mandated GMP regulations and/or guidelines. Such re-validation may be costly and time-consuming. It may be difficult or impossible for us to quickly find replacement manufacturers on acceptable terms, if at all.

- Our contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to produce, store and distribute our products successfully.

The successful commercialization of our product candidates, if approved, will depend on achieving market acceptance and we may not be able to gain sufficient acceptance to generate significant revenue.

Even if our product candidates are successfully developed and receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors such as private insurers or governments and other funding parties and the medical community. The degree of market acceptance for any of our products will depend on a number of factors, including:

- demonstration of the clinical efficacy and safety of our products;

- the prevalence and severity of any adverse side effects;

- limitations or warnings contained in the product s approved labeling;
- cost-effectiveness and availability of acceptable pricing;
- competitive product profile versus alternative treatment methods and the superiority of alternative treatment or therapeutics;
- the effectiveness of marketing and distribution methods and support for the products; and
- coverage and reimbursement policies of government and third-party payors to the extent that our products could receive regulatory approval but not be approved for coverage by or receive adequate reimbursement from government and quasi-government agencies or other third-party payors.

Disease indications may be small subsets of a disease that could be parsed into smaller and smaller indications as different subsets of diseases are defined. This increasingly fine characterization of diseases could have negative consequences, including creating an approved indication that is so small as not to have a viable market for us. If future technology allows characterization of a disease in a way that is different from the characterization used for large pivotal studies, it may make those studies invalid or reduce their usefulness, and may require repeating all or a portion of the studies. Future technology may supply better prognostic ability which could reduce the portion of patients projected to need a new therapy. Even after being cleared by regulatory authorities, a product may later be shown to be unsafe or not to have its purported effect, thereby preventing its widespread use or requiring withdrawal from the market.

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If we fail to obtain coverage and adequate reimbursement for our products, our revenue-generating ability will be diminished and there is no assurance that the anticipated market for our products will be sustained.

We believe that there will be many different applications for products successfully derived from our technologies and that the anticipated market for products under development will continue to expand. However, due to competition from existing or new products and the yet-to-be established commercial viability of our products, no assurance can be given that these beliefs will prove to be correct. Physicians, patients, formularies, payors or the medical community in general may not accept or utilize any products that we or our collaborative partners may develop. Other drugs may be approved during our clinical testing which could change the accepted treatments for the disease targeted and make our compound obsolete.

Our and our collaborators ability to commercialize our products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for such products and related treatments will be available from governmental health payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. No assurance can be given that third-party coverage and adequate reimbursement will be available that will allow us to maintain price levels sufficient for the realization of an appropriate return on our investment in product development.

Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States, in Canada and in many other countries, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to varying degrees of government control. Healthcare reform and controls on healthcare spending may limit the price we charge for any products and the amounts thereof that we can sell. In particular, in the United States, the federal government and private insurers have changed and have considered ways to change, the manner in which healthcare services are provided. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the healthcare industry. The provisions of PPACA of importance to our product candidates include the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, to 23.1% and 13.0% of the average manufacturer price for most branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for a manufacturer s outpatient drugs to be covered under Medicare Part D;

- extension of a manufacturer s Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the federal poverty level beginning in 2014, thereby potentially increasing a manufacturer s Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- new requirements under the federal Open Payments program and its implementing regulations (as described below);

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

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In addition, other legislative changes have been proposed and adopted since PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

We anticipate that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the reimbursement we may receive for any approved product. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 requires the Centers for Medicare & Medicaid Services, or CMS, to reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which in turn will serve as a base for 2014 and subsequent years. CMS also recently proposed to re-examine payment amounts for tests reimbursed under the Medicare clinical laboratory fee schedule due to changes in technology and, in addition, proposed to bundle the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting. The proposals would replace the current methodology for certain tests and, if adopted, the changes would begin to go into effect January 1, 2014 for some codes. Levels of reimbursement may be impacted by current and future legislation, regulation or reimbursement policies of third-party payors in a manner that may harm the demand and reimbursement available for our products, including our companion diagnostic, which in turn, could harm our future product pricing and sales. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

Competition in our targeted market area is intense and this field is characterized by rapid technological change. Therefore developments by competitors may substantially alter the predicted market or render our product candidates uncompetitive.

There are several hundred drugs in clinical development today in the area of oncology therapeutics. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. In the oncology market, our major competitors include, but are not limited to: Amgen Inc.; ArQule Inc. and its partners Kyowa Hakko Kirin Pharma Inc. and Daiichi Sankyo Company Limited; Aveo Pharmaceuticals Inc.; Bristol-Myers Squibb Company; Exelixis Inc.; F. Hoffman-LaRoche Ltd.; GlaxoSmithKline PLC; Novartis AG; and Pfizer, among others.

Many companies have filed, and continue to file, patent applications in oncology which may or could affect our program. Some of these patent applications may have already been allowed or issued, and others may issue in the future. These companies include, but are not limited to: Bristol-Myers Squibb; Compugen Limited; Exelixis; GlaxoSmithKline; Novartis; and Pfizer. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed, and additional patents granted, in the future, as well as additional research and development programs expected in the future.

In addition to companies that have HDAC inhibitors or kinase inhibitors addressing oncology indications, our competition also includes hundreds of private and publicly traded companies that operate in the area of oncology but have therapeutics with different mechanisms of action. The oncology market in general is highly competitive with over 1,000 molecules currently in clinical development.

Developments by others may render our products or technologies non-competitive or obsolete or we may not be able to keep pace with technological developments. Our competitors may have developed or may be developing technologies which may be the basis for competitive

products. Some of these products may prove to be more effective and less costly than the products developed or being developed by us. Our competitors may obtain regulatory approval for their products more rapidly than we do which may change the standard of care in the indications we are targeting, rendering our technology or products non-competitive or obsolete. Others may develop treatments or cures superior to any therapy we are developing or will develop. Moreover, alternate, less toxic forms of medical treatment may be developed which may be competitive with our products.

Many of the organizations which could be considered to be our competitors have substantially more financial and technical resources, more extensive discovery research, preclinical research and development capabilities and greater manufacturing, marketing, distribution, production and human resources than we do. Many of our current or potential competitors have more experience than us in research, preclinical testing and clinical trials, drug commercialization, manufacturing and marketing, and in obtaining domestic and foreign regulatory approvals. In addition, failure, unacceptable toxicity, lack of sales or disappointing sales or other issues regarding competitors products or processes could have a material adverse effect on our product candidates, including our clinical candidates or our lead compounds. Established

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

We will not be able to successfully commercialize our product candidates without establishing sales and marketing capabilities internally or through collaborators.

We currently have no sales and marketing staff. We may not be able to find suitable sales and marketing staff and collaborators for all of our product candidates. We have no prior experience in the marketing, sale and distribution of pharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any collaborators may not be adequate or successful or could terminate or materially reduce the effort they direct to our products. The development of a marketing and sales capability will require significant expenditures, management resources and time. The cost of establishing such a sales force may exceed any potential product revenue, or our marketing and sales efforts may be unsuccessful. If we are unable to develop an internal marketing and sales capability in a timely fashion, or at all, or if we are unable to enter into a marketing and sales arrangement with a third party on acceptable terms, we may be unable to successfully develop and seek regulatory approval for our product candidates and/or effectively market and sell approved products, if any.

We are subject to competition for our skilled personnel and may experience challenges in identifying and retaining key personnel that could impair our ability to conduct our operations effectively.

Our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel. If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy. Although we have not experienced problems attracting and retaining highly qualified personnel in the recent past, our industry has experienced a high rate of turnover of management personnel in recent years. 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, especially Charles M. Baum, M.D., Ph.D., our President and Chief Executive Officer, Mark J. Gergen, our Executive Vice President and Chief Operations Officer, Isan Chen, M.D., our Executive Vice President and Chief Medical and Development Officer, James Christensen, Ph.D. our Vice President of Research, and Jamie A. Donadio, our Vice President of Finance, whose services are critical to the successful implementation of our product candidate acquisition, development and regulatory strategies, as well as the management of our financial operations. We are not aware of any present intention of any of these individuals to leave our Company. In order to induce valuable employees to continue their employment with us, we have provided stock options that vest over time. The value to employees of stock options that vest over time is significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could harm our business, financial condition and prospects. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

We may also experience growth in the number of our employees and the scope of our operations, especially in clinical development. This growth will place a significant strain on our management, operations and financial resources and we may have difficulty managing this future potential growth. No assurance can be provided that we will be able to attract new employees to assist in our growth. Many of the other 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. We also may employ consultants or part-time and contract employees. There can be no assurance that these individuals are retainable. While we have been able to attract and retain skilled and experienced personnel and consultants in the past, no assurance can be given that we will be able to do so in the future.

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Our recently announced closure of our Canadian and New Jersey operations and related reduction in employees may disrupt our business, and we may not be able to adequately replace lost functionality through planned additional hiring at our San Diego facility or use of third-party service providers.

In connection with the Arrangement completed on June 28, 2013, we relocated our corporate headquarters from Montreal, Canada to San Diego, California. Since relocating to San Diego, we have maintained operations in our Canadian office. In addition to the ongoing operations in our Canadian office, we also maintain facilities in Princeton, New Jersey. On October 1, 2013, we announced our intention to close our New Jersey operations as of October 31, 2013 and to transition our Canadian operations to our San Diego offices over the next three to six months. In connection with these efforts, there will be a reduction in force of approximately 27 employees in our Montreal and Princeton offices, or approximately 75% of our workforce. We plan to partially offset this reduction in force by hiring additional personnel in our San Diego office and by engaging third-party service providers to perform certain functions. However, we may not be able to attract and retain the type and number of employees we desire in San Diego, or do so on our planned timeline. During this transition period, we may incur disruptions in our business, including from the loss of functionality we currently maintain in our Montreal and Princeton facilities. In addition, we may be unable to realize the efficiencies we are seeking by consolidating our operations in a single office in San Diego. If we are unable to realize such efficiencies or attract and retain qualified personnel in San Diego and effectively outsource certain other functions to third-party service providers, our operations and ability to execute our business plan would be adversely affected.

Our current and future relationships with customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients rights are and will be applicable to our business. Our current and future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which we sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid;

- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal Open Payments program, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children s Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments or other transfers of value made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members, with data collection beginning on August 1, 2013, requirements for manufacturers to submit reports to CMS by March 31, 2014 and the 90th day of each subsequent calendar year, and disclosure of such information to be made by CMS on a publicly available website beginning in September 2014; and

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- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exceptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute such that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. Moreover, PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. To the extent that any of our product candidates is ultimately sold in countries other than the United States, we may be subject to similar laws and regulations in those countries. If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, imprisonment, exclusion from participation in government healthcare programs, and the curtailment or restructuring of our operations, any of which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including any of our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusion from participation in government healthcare programs, which could also materially affect our business.

We may become subject to the risk of product liability claims.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. Human therapeutic products involve the risk of product liability claims and associated adverse publicity. Currently, the principal risks we face relate to patients in our clinical trials, who may suffer unintended consequences. Claims might be made by patients, healthcare providers, pharmaceutical companies or others. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates, if approved. Even successful defense would require significant financial and management resources.

Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates;
- 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 from product sales; and
- the inability to commercialize any our product candidates, if approved.

We may not have or be able to obtain or maintain sufficient and affordable insurance coverage, and without sufficient coverage any claim brought against us could have a materially adverse effect on our business, financial condition or results of operations. We run clinical trials through investigators that could be negligent through no fault of our own and which could affect patients, cause potential liability claims against us and result in delayed or stopped clinical trials. We are required in many cases by contractual obligations to indemnify collaborators, partners, third-party contractors, clinical

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investigators and institutions. These indemnifications could result in a material impact due to product liability claims against us and/or these groups. We currently carry $10 million in product liability insurance, which we believe is appropriate for our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our business involves the controlled use of hazardous materials and as such we are subject to environmental and occupational safety laws. Continued compliance with these laws may incur substantial costs and failure to maintain compliance could result in liability for damages that may exceed our resources.

Our preclinical research, manufacturing and development processes involve the controlled use of hazardous and radioactive materials. We are subject to federal, provincial and local laws and regulations governing the use, manufacture, storage, handling and disposal of such materials and certain waste products. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. The risk of accidental contamination or injury from these materials cannot be completely eliminated. In the event of such an accident, we could be held liable for any damages that result, and any such liability could exceed our resources. We may not be adequately insured against this type of liability. We may be required to incur significant costs to comply with environmental laws and regulations in the future, and our operations, business or assets may be materially adversely affected by current or future environmental laws or regulations.

We may have to dedicate resources to the settlement of litigation.

Securities legislation in both the United States and Canada makes it relatively easy for stockholders to sue. This could lead to frivolous law suits which could take substantial time, money, resources and attention or force us to settle such claims rather than seek adequate judicial remedy or dismissal of such claims.

If we are required to defend patent infringement actions brought by third parties, or if we sue to protect our own patent rights or otherwise to protect our proprietary information and to prevent its disclosure, we may be required to pay substantial litigation costs and managerial attention may be diverted from business operations even if the outcome is in our favor. If we are required to defend our patents or trademarks against infringement by third parties, we may be required to pay substantial litigation costs and managerial attention and financial resources may be diverted from our research and development operations even if the outcome is in our favor.

We may be vulnerable to disruption, damage and financial obligation as a result of system failures.

Despite the implementation of security measures, any of the internal computer systems belonging to us, our collaborators or our third party service providers are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failure. Any system failure, accident or security breach that causes interruptions in our own, in collaborators or in third party service vendors operations could result in a material disruption of our drug discovery programs. To the extent that any disruption or security

breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liability, our drug discovery programs may be adversely affected and the further development of our product candidates may be delayed. Furthermore, we may incur additional costs to remedy the damages caused by these disruptions or security breaches.

Risks Relating to Our Intellectual Property

We may not obtain adequate protection for our product candidates through patents and other intellectual property rights and as such our competitive advantage in the marketplace may be compromised.

Our success depends, in part, on our ability to secure and protect our patents, trade secrets, trademarks and other intellectual property rights and to operate without infringing on the proprietary rights of others or having third parties circumvent the rights that we own or license. We have filed and are actively pursuing patent applications in the United States, Canada, Japan, Europe and other major markets via the Patent Cooperation Treaty or directly in countries of interest. The patent positions of healthcare companies, universities and biopharmaceutical companies, including ours, are uncertain and involve complex questions of law and fact for which important legal issues may remain unresolved. Therefore, there is no assurance that our pending patent applications will result in the issuance of patents or that we will develop additional

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proprietary products which are patentable. Moreover, patents issued or to be issued to us may not provide us with any competitive advantage. Further, if the patent applications we hold or in-license with respect to our programs, product candidates and companion diagnostic fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products.

Our patents may be challenged by third parties in patent litigation. In addition, it is possible that third parties with products that are very similar to ours will circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts. There are no assurances that our patent counsel, lawyers or advisors have given us correct advice or counsel. Opinions from such patent counsel or lawyers may not be correct or based on incomplete facts. We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor s technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products. The steps we have taken to protect our intellectual property may not prevent the misappropriation of our proprietary information and technologies, particularly in foreign countries where laws or law enforcement practices may not protect proprietary rights as fully as in Canada, the United States or Europe. Unauthorized disclosure of our proprietary information could also harm our competitive position. We could also inadvertently use our collaborators data inappropriately which could lead to liability. We may file patent applications but have claims restricted or we may not be able to supply sufficient data to satisfy a patent office to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims. Alternatively, it is possible that we may not receive any patent protection from an application.

Maintaining our patents and applications requires timely payment of fees and other associated costs in the countries of filing, and we could inadvertently abandon a patent or patent application (or trademark or trademark application) due to non-payment of fees, or as a result of a failure to comply with filing deadlines or other requirements of the prosecution process, resulting in the loss of protection of certain intellectual property rights in a certain country. Alternatively, we, our collaborators or our patent counsel may take action resulting in a patent or patent application becoming abandoned which may not be able to be reinstated, or if reinstated, may suffer patent term adjustments. Any of these outcomes could hurt our ability to gain full patent protection for our products. Registered trademarks in Canada, the United States and other countries that belong to us are subject to the same risks as described above for patents and patent applications.

Many of our collaboration agreements are complex and may call for licensing or cross-licensing of potentially blocking patents, know-how or intellectual property. Due to the potential overlap of data, know-how and intellectual property rights there can be no assurance that one of our collaborators will not dispute our right to send data or know-how or other intellectual property rights to third parties and this may potentially lead to liability or termination of a program. There are no assurances that the actions of our collaborators would not lead to disputes or cause us to default with other collaborators. We cannot be certain that a collaborator will not challenge the validity of licensed patents.

We cannot be certain that any country s patent and/or trademark office will not implement new rules which could affect how we draft, file, prosecute and/or maintain patents and patent applications. We cannot be certain that increasing costs for drafting, filing, prosecuting and maintaining patent applications and patents will not restrict our ability to file for patent protection, or to prosecute applications through to grant. We may be forced to abandon or return the rights to specific patents due to a lack of financial resources. There is no assurance that we could enter into licensing arrangements at a reasonable cost, or develop or obtain alternative technology in respect of patents issued to third parties that incidentally cover our products. Any inability to secure licenses or alternative technology could result in delays in the introduction of some of

our products or even lead to prohibition of the development, manufacture or sale of certain products by us.

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We have filed applications for trademark registrations in connection with our product candidates in various jurisdictions, including the United States. We intend to file further applications for other possible trademarks for our product candidates. No assurance can be given that any of our trademark applications will be registered in the United States or elsewhere, or that the use of any registered or unregistered trademarks will confer a competitive advantage in the marketplace. Furthermore, even if we are successful in our trademark registrations, the FDA and regulatory authorities in other countries have their own process for drug nomenclature and their own views concerning appropriate proprietary names. No assurance can be given that the FDA or any other regulatory authority will approve of any of our trademarks or will not request reconsideration of one of our trademarks at some time in the future. The loss, abandonment, or cancellation of any of our trademarks or trademark applications could negatively affect the success of the product candidates to which they relate.

Moreover, some of our know-how and technology which is not patented or not patentable may constitute trade secrets. Therefore, we require our consultants, advisors and collaborators to enter into confidentiality agreements and our employees to enter into invention, non-disclosure and non-compete agreements. However, no assurance can be given that such agreements will provide for a meaningful protection of our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure of information. Furthermore, we cannot provide assurance that any of our employees, consultants, contract personnel or collaborators, either accidentally or through willful misconduct, will not cause serious impact to our programs and/or our strategy. All of our employees have signed confidentiality agreements, but there can be no assurance that they will not inadvertently or through their misconduct give trade secrets away.

Third-party intellectual property infringement claims may result in a reduction in the scope of our patent protection and competitive exclusivity with respect to our product candidates. Patent litigation, including defense against third-party intellectual property claims, may result in us incurring substantial costs.

Patent applications which may relate to or affect our business may have been filed by others. Such patent applications or patents resulting therefrom may conflict with our technologies, patents or patent applications and reducing the scope of our patent protection. Such events could cause us to stop or change the course of our research and development or modify our intellectual property strategies. We could also become involved in interference proceedings in connection with one or more of our patents or patent applications to determine priority of invention. There can be no guarantees that an interference proceeding would be successful or that such an outcome could be reversed on appeal. 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. Our defense of such interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.

No assurance can be given that our patents, once issued, would be declared by a court to be valid or enforceable, or that we would not be found to infringe a competitor s patent.

Third parties may assert that we are using their proprietary information without authorization. Third parties may also have or obtain patents and may claim that technologies licensed to or used by us infringe their patents. Because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates or companion diagnostic may infringe, or which such third parties claim are infringed by the use of our technologies. If any third-party patents are held by a court of competent jurisdiction to cover any aspect of our product candidates, including the formulation or method of use of such product candidate, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. In any such case, such a license may not be available on commercially reasonable terms or at all. In addition, any legal action that seeks damages or an injunction to stop us from carrying on our commercial activities relating to the affected technologies could subject us to monetary liability. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources.

Parties making claims against us for infringement of their intellectual property rights may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and expense, which could force us to cease commercialization of one or more of our product candidates, or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be req